



Health Technology Assessment Clinical Committee Meeting Program Update

Leah Hole-Curry, JD

Health Technology Assessment

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Presentation Overview

- **HTA Program Overview**
- **HTA Program Updates**
 - Topics

Today's Topic

- ABA Therapy for Autism

Governor Gregoire's strategy : Improve quality in health care

- **Governor Gregoire's five point plan to improve health care (2005)**
 - *Emphasize evidence based health care*
 - Create more transparency in the health care system
 - Promote prevention, healthy lifestyles, and healthy choices
 - Better managed chronic care
 - Make better use of information technology
- **WA State Legislature and Blue Ribbon Commission (2006)**
 - Goals set for 2012 including use of evidence based medicine
- **Collaboration of Programs across State purchasing –**
 - Total of about 450,000 beneficiaries and 3.5 billion purchased
 - Health Care Authority – Public Employees and subsidized low income (Basic Health, **Uniform Medical Plan**, PEBB)
 - **Medicaid** Purchasing Agency – federal/state low income health care program with **fee for service** and managed care plans
 - Labor and Industries – **Worker's compensation** program
 - Department of Corrections – Correctional health care

Why Health Technology Assessment?

- **Part of an overall strategy**
- **Medical technology is a primary driver of cost**
 - The development and diffusion of medical technology are primary factors in explaining the persistent difference between health spending and overall economic growth.
 - Some health experts arguing that new medical technology may account for about one-half or more of real long-term spending growth.

Kaiser Family Foundation, March 2007: **How Changes in Medical Technology Affect Health Care Costs**

- **Medical Technology has quality gaps**
 - Medical technology diffusing without evidence of improving quality Highly correlated with misuses, overutilization, underutilization.

Cathy Schoen, Karen Davis, Sabrina K.H. How, and Stephen C. Schoenbaum, "U.S. Health System Performance: A National Scorecard," *Health Affairs*, Web Exclusive (September 20, 2006): w459

KEY HTA Products

Pay for What Works: Better Information is Better health

- **Transparency**: Publish topics, criteria, reports, open meeting
- **Technology Assessment Report**: Formal, systematic process to review appropriate healthcare technologies.
- **Independent Coverage decision**: Committee of practicing clinicians make decisions that are scientifically based, transparent, and consistent across state health care purchasing agencies.

Key focus questions:

- Is it safe?
- Is it effective?
- Does it provide value (improve health outcomes)?

HTA Program Elements

1. HCA Administrator Selects Technology

Nominate, Review, Public Input, Prioritize

Semi-annual

2. Vendor Produce Technology Assessment Report

Key Questions and Work Plan, Draft, Comments, Finalize

2-8 Months

3. Clinical Committee makes Coverage Determination

Review report, Public hearing

Meet Quarterly

4. Agencies Implement Decision

Implements within current process unless statutory conflict

Evidence for use in Policy Decisions

Different Data Sources

■ **Efficacy**

- How technology functions in “best environments”
 - Randomized trials-distinguish technology from other variables
 - Meta-analysis

■ **Effectiveness**

- How technology functions in “real world”
 - Population level analyses
 - Large, multicenter, rigorous observational cohorts (consecutive pts/objective observers)

■ **Safety**

- Variant of effectiveness
 - Population level analyses
 - Case reports/series, FDA reports

■ **Cost**

- Direct and modeled analysis
 - Administrative/billing data (charge vs cost)

■ **Context**

- Mix of historic trend, utilization data, beneficiary status, expert opinion

- **Clinical Committee Decision must give greatest weight to most valid and reliable evidence**
 - Objective Factors for evidence consideration
 - Nature and Source of evidence
 - Empirical characteristics of the studies or trials upon which evidence is based
 - Consistency of outcomes with comparable studies
 - Additional evaluation factors
 - Recency (date of information)
 - Relevance (applicability of the information to the key questions presented or participating agency programs and clients)
 - Bias (presence of conflict of interest or political considerations)

- ABA Therapy for Autism
- Sleep Apnea Diagnosis and Treatment
- Femoro-acetabular surgery for hip impingement syndrome
- Positron Emission Tomography (PET) scans for Lymphoma
- Microprocessor controlled Prosthetics – lower limb
- Bone graft products (autograft, allograft and synthetic)
- Osteoarticular Transfer System Cartilage Surgery (OATS)
- CT/MR for Pelvic and Abdomen
- Elective Cesarean Section
- Stereotactic radiosurgery
- Robotic assisted surgical devices (e.g. Davinci, Zeus)
- Upper Endoscopy for GERD

**Washington State Health Care Authority, HTA Program
Key Questions and Background
ABA Therapy for Autism Spectrum Disorder**

Introduction

HTA has selected ABA Therapy for Autism Spectrum Disorder to undergo a health technology assessment where an independent vendor will systematically review the evidence available on the safety, efficacy, and cost-effectiveness. HTA posted the topic and gathered public input on all available evidence. HTA published the Draft Key Questions to gather input about the key questions and any additional evidence to be considered in the evidence review, and will review the public comments submitted and finalize the key questions. Key questions guide the development of the draft evidence report.

In this case, a federal research agency, AHRQ, also selected this topic. AHRQ previously posted for public comment its key questions and has just released a draft report. HTA strives to make economical use of state resources and to not duplicate other systematic reviews where current reports meet our statutory mandate and are timely.

Therefore, HTA requested comments on the draft key questions that were posed in the AHRQ report, and comments on whether any additional questions would be needed to meet HTA's specific purposes. Regardless of outcome, HTA strongly encouraged stakeholders interested in this topic to also participate in the AHRQ review and comment process. The AHRQ comment form on the draft report was open until August 6th and could be accessed at:

<http://www.effectivehealthcare.ahrq.gov/index.cfm/research-available-for-comment/comment-draft-reports/?pageaction=displayDraftCommentForm&topicid=106&productID=478>

Proposed Key Questions *(As specified in AHRQ report)*

Source:

<http://www.effectivehealthcare.ahrq.gov/ehc/products/106/366/Therapies%20for%20Autism%20Spectrum%20Disorder%20%282-9-2010%29.pdf>

KQ1: Among children ages 2-12 with ASD, what are the short and long-term effects of available behavioral, educational, family, medical, allied health, or CAM treatment approaches? Specifically,

KQ1a: What are the effects on core symptoms (e.g. social deficits, communication deficits and repetitive behaviors), in the short term (≤ 6 months)?

KQ1b: What are the effects on commonly associated symptoms (e.g. motor, sensory, medical, mood/anxiety, irritability, and hyperactivity) in the short term (≤ 6 months)?

KQ1c: What are the longer-term effects (> 6 mos) on core symptoms (e.g. social deficits, communication deficits and repetitive behaviors)?

KQ1d: What are the longer-term effects (> 6 mos) on commonly associated symptoms (e.g. motor, sensory, medical, mood/anxiety, irritability, and hyperactivity)?

KQ2: Among children ages 2-12, what are the modifiers of outcome for different treatments or approaches?

KQ2a: Is the effectiveness of the therapies reviewed affected by the frequency, duration, and intensity of the intervention?

KQ2b: Is the effectiveness of the therapies reviewed affected by the training and/or experience of the individual providing the therapy?

KQ2c: What characteristics, if any, of the child modify the effectiveness of the therapies reviewed?

KQ2d: What characteristics, if any, of the family modify the effectiveness of the therapies reviewed?

KQ3: Are there any identifiable changes early in the treatment phase that predict treatment outcomes?

KQ4: What is the evidence that effects measured at the end of the treatment phase predict long term functional outcomes?

KQ5: What is the evidence that specific intervention effects measured in the treatment context generalize to other contexts (e.g., people, places, materials)?

KQ6: What evidence supports specific components of treatment as driving outcomes, either within a single treatment or across treatments?

KQ7: What evidence supports the use of a specific treatment approach in children under the age of 2 who are at high risk of developing autism based upon behavioral, medical, or genetic risk factors?

PICOTS (From AHRQ Report)

Population. Children ages 2 – 12 who are diagnosed with an autism spectrum disorder (ASD) and children under age 2 at risk for diagnosis of an ASD

Interventions. Behavioral interventions, including variations of applied behavior analysis as well as developmentally-based models such as DIR/Floortime, among others; educational interventions, including the TEACCH program; allied health interventions, including occupational, physical, and speech therapy; medical interventions, including prescription and non-prescription treatments; and CAM approaches, including music therapy and nutritional therapies intended to modify the core symptoms of ASD

Comparators. No treatment, placebo, or comparative interventions from intervention list or combinations of interventions.

Outcomes and adverse events.

Primary outcomes.

- Changes in short-term targeted outcome areas, including social skills/interaction, language and communication, repetitive and other maladaptive behaviors, psychological distress, adaptive skills development and academic skills development

Technology Background

Technology: Autism Spectrum Disorders (ASD) are common neurodevelopmental disorders, with an estimated prevalence of one in 110 children in the United States. Individuals with ASD have significant impairments in social interaction, behavior, and communication. Children with ASD may also have impaired cognitive skills and sensory perception. The expression and severity of symptoms of ASD differ widely, and treatments include a range of behavioral, psychosocial, educational, medical, and complementary approaches that vary by a child's age and developmental status. The goals of treatment for ASD focus on improving core deficits in communication, social interactions, or restricted behaviors, with the idea that changing these fundamental deficits may help children develop greater functional skills and independence. Individual goals for treatment will vary for different children, and may include combinations of medical and related therapies, behavioral therapies, educational therapies, allied health therapies and complementary and alternative medicine (CAM) therapies. Important questions remain about the efficacy and safety of therapies, including Applied Behavioral Therapy, which groups of individuals with ASD may benefit, and whether a combination of medical and other therapies is necessary.

Participant Conflict of Interest Guideline

Introduction

The HTCC Workgroup is a public service workgroup established to safeguard the public interest by identifying medical tests and treatments where evidence shows they are safe, effective, and cost-effective. Balance, independence, objectivity and scientific rigor are a basis for public trust and crucial to the credibility and integrity of decisions.

Guiding Principle

Conflict of Interest decisions must be disclosed and balanced to ensure the integrity of decisions while acknowledging the reality that interests, and sometimes even conflicting interests, do exist. Individuals that stand to gain or lose financially or professionally, or have a strong intellectual bias need to disclose such conflicts.

For example, the fact that a member or stakeholder is a health care provider that may use a service under review creates a potential conflict. However, clinical and practical knowledge about a service is also useful, and may be needed in the decision making.

Procedure

Declaration of real or potential conflicts of interest, professional, intellectual, or financial is required prior to membership or provision of written or verbal commentary. Participants must sign a conflict of interest form; stakeholders providing comment must disclose conflicts.

The HTCC Chair or HCA Administrator shall make a decision, in his/her sole discretion, as to whether a conflict of interest rises to the level that participation by the conflicted participant could result in a loss of public trust or would significantly damage the integrity of the decision.

HCA defines conflict of interest as any situation in which a voting member or anyone who provides written or verbal testimony regarding products, services, or technologies discussed or voted on during the workgroup meeting, has a relationship with a manufacturer of any commercial products and / or provider of services discussed or voted on during the meeting. Relationship extends to include immediate family member(s) and / or any entity in which the member or person testifying may have an interest.

A relationship is considered as:

1. Receipt or potential receipt of anything of monetary value, including but not limited to, salary or other payments for services such as consulting fees or honoraria in excess of \$10,000.
2. Equity interests such as stocks, stock options or other ownership interests in excess of \$10,000 or 5% ownership, excluding mutual funds and blinded trusts.
3. Status of position as an officer, board member, trustee, owner or employee of a company or organization representing a company, association or interest group.
4. Loan or debt interest; or intellectual property rights such as patents, copyrights and royalties from such rights.
5. Manufacturer or industry support of research in which you are participating.
6. Any other relationship that could reasonably be considered a financial, intellectual, or professional conflict of interest.
7. Representation: if representing a person or organization, include the organization's name, purpose, and funding sources (e.g. member dues, governmental/taxes, commercial products or services, grants from industry or government).
8. Travel: if an organization or company has financially paid your travel accommodations (e.g. airfare, hotel, meals, private vehicle mileage, etc).



Disclosure

Any unmarked topic will be considered a "Yes"

	Potential Conflict Type	Yes	No
1.	Salary or payments such as consulting fees or honoraria in excess of \$10,000		X
2.	Equity interests such as stocks, stock options or other ownership interests		X
3.	Status of position as an officer, board member, trustee, owner		X
4.	Loan or intellectual property rights	X	
5.	Research funding		X
6.	Any other relationship		X

If yes, list name of organizations that relationship(s) are with and for #6, describe other relationship:

I am one of the co-authors on the article "Randomized, Controlled Trial of an Intervention for Toddlers with Autism: The Early Start Denver Model" (Dawson et al, 2010); however, I am not an author on the book, "Early Start Denver Model for Young Children with Autism: Promoting Language, Learning, and Engagement" (Rogers & Dawson, 2009) and I have no financial interest in the book or intervention training.

	Potential Conflict Type	Yes	No
7.	Representation: if representing a person or organization, include the name and funding sources (e.g. member dues, governmental/taxes, commercial products or services, grants from industry or government).		X

7. If yes, Provide Name and Funding Sources: _____

	Potential Conflict Type	Yes	No
8.	Travel: if an organization or company has financially paid your travel accommodations (e.g. airfare, hotel, meals, private vehicle mileage, etc).		X

8. If yes, Provide Name of Organization / Company and Disclose Travel Accommodations:



If you believe that you do not have a conflict but are concerned that it may appear that you do, you may **attach additional sheets** explaining why you believe that you should not be excluded.

I certify that I have read and understand this Conflict of Interest Form and that the information I have provided is true, complete, and correct as of this date.

X

[Redacted Signature]

Signature

6/2/11

Date

Amy L. Donaldson

Print Name

FOR QUESTIONS: Denise Santoyo, Health Care Authority, 360-923-2742,
PO Box 42712, Olympia, WA 98504-2712

Amy L. Donaldson, Ph.D., CCC-SLP is an Assistant Professor in the Department of Speech & Hearing Sciences at Portland State University (PSU). Her research focuses on the assessment and intervention of social communication skills in children with autism spectrum disorder (ASD) within the natural environment. Dr. Donaldson is currently part of a multi-university team that is investigating the effects of a preschool-based, joint attention and symbolic play intervention for children with ASD. She is also examining the efficacy of sibling-mediated intervention for young children with ASD. Prior to joining PSU, Dr. Donaldson was a Research Assistant Professor at the University of Washington (UW), where she also completed her doctorate. At UW, she was part of an interdisciplinary team investigating the effects of an intensive developmental behavioral intervention for toddlers with ASD (the Early Start Denver Model). Dr. Donaldson received her Master of Science in Speech-Language Pathology at Gallaudet University in Washington, D.C. She has been working clinically with children demonstrating communication challenges, and their families, for over 16 years.

ABA Therapy - Scheduled Public Comments (5 minutes per presenter)

#	Name	Representing	COI	PPT
1	Arzu Forough	Washington Autism Advocacy	Yes	No

Disclosure

Any unmarked topic will be considered a "Yes"

	Potential Conflict Type	Yes	No
1.	Salary or payments such as consulting fees or honoraria in excess of \$10,000		X
2.	Equity interests such as stocks, stock options or other ownership interests		X
3.	Status or position as an officer, board member, trustee, owner		X
4.	Loan or intellectual property rights		X
5.	Research funding		X
6.	Any other relationship, including travel arrangements		X

If yes, list name of organizations that relationship(s) are with and for #6, describe other relationship:

	Potential Conflict Type	Yes	No
7.	Representation: if representing a person or organization, include the name and funding sources (e.g. member dues, governmental/taxes, commercial products or services, grants from industry or government).		X

7. If yes, Provide Name and Funding Sources: I'm the founder of Washington Autism Advocacy, but I'm representing my children. Also, WAA does not have any funding sources, such as member dues, government/taxes/commercial products or services or grants from industry or government. It is 100% volunteer run.

If you believe that you do not have a conflict but are concerned that it may appear that you do, you may **attach additional sheets** explaining why you believe that you should not be excluded.

I certify that I have read and understand this Conflict of Interest Form and that the information I have provided is true, complete, and correct as of this date.


5.2.11
Arzu Forough

Signature
Date
Print Name

FOR QUESTIONS: Denise Santoyo, Health Care Authority, 360-923-2742,
PO Box 42712, Olympia, WA 98504-2712

ABA COVERAGE COMMENTS FOR GOVERNMENT REVIEW

ERIC BRECHNER, MICROSOFT EMPLOYEE AND PARENT

In November of 1998, ten Microsoft employees wrote to the Microsoft Chief Operating Officer and the Director of Human Resources (see *Excerpt from the letter to HR*). We talked about how the company, given the right guidelines, can cover behavioral intervention responsibly and practically. We talked about the impact to our families.

In January 2001, Microsoft introduced coverage for autism therapy, like Applied Behavioral Analysis (ABA). This coverage has been enhanced three times since.

- Removal of age limits in 2002
- Increase in the number of consultant visits in 2008
- Removal all lifetime limits in 2011

Microsoft regularly enhanced coverage because the coverage paid for itself within three years, increased employee productivity, helped with recruiting, and improved employee retention.

The precise impact of the Microsoft autism benefit is difficult to measure due to privacy regulations. Nonetheless in 2006, Microsoft employees decided to anonymously survey themselves.

- 60 total respondents—roughly half of the Microsoft autism distribution list at the time
- 50% considered the autism therapy benefit an “important factor” in their decision to join Microsoft
- 60% considered the autism therapy benefit an “important factor” for retention
- 19% indicated they were likely to leave if the autism therapy benefit expired

In 2006, the U.S. Department of Labor reported the average length someone stays at one job in the US was 3 to 5 years. In 2011, 6 of the 10 Microsoft employees who wrote about their autistic children are still at Microsoft 13 years later.

Although Microsoft HR reports that the autism therapy benefit provides a return on investment of about 7% after three years (roughly 70% of which is due to productivity gains), the literature indicates potential gains of 250-350% over 20 years¹. However, these gains can only be realized though broad adoption of autism therapy. When government gets involved, we can all achieve that 250-350% return.

¹ Jacobson et al. (1998) ~\$250,000 per child 3-22 years; Hildebrand (1999) ~\$350,000 per child 3-22 years

EXCERPT FROM THE LETTER TO MICROSOFT HR – NOVEMBER, 1998

Microsoft Benefits mentions five primary concerns. As a Microsoft stockholder, I am quite sensitive to the need to cover only narrowly prescribed rehabilitative therapies provided by licensed or otherwise credentialed providers. Doing otherwise exposes the company to excessive liability and expense. I believe the company, given the right guidelines, can cover behavioral intervention responsibly and practically.

Allow me to respond to each of Microsoft Benefits' primary concerns:

- Microsoft healthcare plan and prevailing benefit industry standards exclude educational therapy from coverage under health care plans

ABA therapy for autistic children is rehabilitative, not educational. The therapy develops basic imitation skills, speech, and the ability to interact with other people in fundamental ways that come naturally to every typically developing child. It is precisely these skills that children with developmental or neurological disorders need to have access to any of the educational services, even special education services, that a school district can provide. There is significant and compelling documented research demonstrating the effectiveness of this behavioral intervention as rehabilitative therapy for developmental and neurological disorders.

- Treatment is provided by unlicensed, non-credentialed graduate students

A credentialed psychologist designs and develops my son's ABA program. She regularly evaluates Peter's progress and trains and supervises the individuals that do the 20-30 hours a week of one-on-one therapy. The individuals she supervises are often students. As with any long-term intensive care, the day to day attention is not given by the credentialed professional, but instead by supervised apprentices. Many covered therapies, such as physical therapy, are done in an identical fashion. The key is that the liability goes back to the supervising licensed and/or credentialed professional.

Since this point is brought up several times, I'd like to make the following comparison. People who suffer strokes or head injuries that result in the loss of communication, cognitive, social, and daily living skills are routinely provided with intensive rehabilitation to re-train those skills. This kind of therapy has many things in common with intensive ABA for children with autism. It's provided 1-on-1 by specially trained, but not credentialed individuals, under the supervision of a credentialed professional. It's intrusive and intensive; it must be done for many hours over extended periods of time to be effective; and more than likely, it couldn't be done properly in a setting like a typical public school program. Insurance pays for much of this kind of rehabilitation, as long as it's prescribed and directed doctors. Autism is also a neurological disorder; the only difference is that, unlike stroke, autism affects brain functioning from birth (or more likely, prior to birth). So instead of re-learning how to function independently in regular environments, like stroke patients, children with autism have to learn how to do that from the get-go, and typical educational services simply don't suffice.

- Lack of regulation for licensed treatment providers

Both Psychology and Neurology are well established and credentialed fields. As long as someone with these credentials is directing the program this should not be an issue.

- Liability issues with treatment provided by unlicensed and/or non-credentialed providers

Again, this should not be an issue when the liability goes back to the supervising licensed and/or credentialed professional.

- ABA is also used to treat other diagnosis and would dramatically affect the Microsoft health care plan

ABA and behavioral intervention in general could be used for many different purposes. This fact is irrelevant to whether or not ABA should be used to treat autism. Many drugs and other treatments

can be used for illegitimate purposes including performance enhancement and recreation. Behavioral intervention should only be covered when a licensed physician, psychologist, or neurologist prescribes it for a developmental or neurological disorder. The key is that a trusted professional is prescribing the behavioral intervention to treat only certain conditions for which it has been proven an effective rehabilitative therapy.

To summarize, if a licensed physician, psychologist, or neurologist prescribes behavioral intervention (ABA) for the treatment of a developmental or neurological disorder, and that treatment is directed by a licensed and/or credentialed professional (Psychologist, Speech Pathologist, Neurologist, etc.), then the therapy should be covered by the Microsoft Benefits plan. I believe by narrowly defining who can receive benefits and under what conditions, Microsoft can responsibly cover this therapy without exposing itself to undue liability or expense.

That said, you should know just how important it is to cover this therapy. Following the advice of the psychologist who diagnosed our son, my wife and I arranged for Peter to receive ABA therapy. In nine months he has gone from a completely silent, unaware, and unresponsive child to a darling little boy. Peter now greets me when he wakes up with, "Hi Daddy!" He kisses me and waves goodbye when I leave. He plays games with his older brother, whom he once didn't even know existed. And at night he snuggles under his covers, looks me right in the eye (something he never could do before), and says, "Night, night. Sweet dreams. I love you. See you in the morning."

Peter still has a long way to go. His speech is delayed, he can't perform many common skills like jumping or catching, he does not interact with others as he does with his immediate family, and he lacks many social and self-help skills that typical children his age have. None the less, when I compare where he was to where he is, tears come to my eyes. It is nothing short of a miracle.

Behavioral intervention has given me back my son from what was once thought a hopeless diagnosis. Although I would spend every penny I have to continue to provide it for him, coverage of this clearly rehabilitative therapy would insure that certified professionals will provide it at the level Peter needs. I have tried to show how this can be done responsibly.

[My son is now 15 years old and is a straight-A student at our local public Junior High School. His speech is no longer delayed, he can jump and catch, and he interacts with his friends in typical yet nerdy ways.]

My 6 year old son is autistic and ABA has done wonders for him after only about a year. He was almost non-verbal when he started and now he can talk in complete sentences, can play games, participates in circle time at school and is far more connected to us. We attribute a large part of this to ABA and expect it to be the key to generalizing him, so I'd like to help if I can, even if it doesn't affect me directly.

Scott Napolitan

June 5, 2011

Health Technology Clinical Committee
c/o Denise Santoyo
Washington State Health Care Authority
Health Technology Assessment

Dear members of the Health Technology Clinical Committee,

RE: REQUEST FOR PUBLIC COMMENT ON THE USE OF APPLIED BEHAVIOR ANALYSIS THERAPY FOR AUTISM

I am writing this letter in response to your request for public comment on the matter of funding for applied behavior analysis therapy for autism. I understand that the committee will be relying on two key reports in making their decision, the Agency for Healthcare Research and Quality (AHRQ) report (April 2011) and the Healthcare Technology Agency (HTA) report (May 2011).

I will first comment on the inconsistencies that I see in each of the reports before making some more general comments about the treatment of individuals with autism.

The AHRQ report: Inconsistent and arbitrary

I found the AHRQ report to be extremely, and inappropriately, conservative in its assessment of the use of applied behavior analysis (ABA) to treat individuals with autism. The following are concerns that I had with the report:

1. It does not acknowledge that ABA is the intervention with the largest data base to support it.
 - a. While there are definitely weaknesses in the literature and more work to be done, this intervention methodology has more evidence to support it than do the medications which the report endorses.
2. One of the criticisms mentioned in the report is that there has not been any research done evaluating the comparison between treatment methodologies.
 - a. While, again, this research does need to be completed, it should be noted that there are no direct studies comparing medication treatments. However, the authors go on to endorse the use of medication with this population anyway. This is a curious double-standard.
 - b. Moreover, despite the authors' contention to the contrary, there are studies that directly address the comparison between treatment methodologies; the results show that intensive early behavioral intervention was superior to an eclectic approach in the treatment of individuals with autism (Howard, Sparkman, Cohen, Green, & Stanisiaw, 2005; Eikeseth, Smith, Jahr, & Eldevik, 2002; Eikeseth, Smith, Jahr, & Eldevik, 2007; Eldevik, Eikeseth, Jahr, & Smith, 2006).
3. It is also important to differentiate between the general field of ABA and the more specific form of intensive and comprehensive early behavioral intervention for individuals with autism.
 - a. The AHRQ report focuses on the latter, but fails to acknowledge the numerous studies that demonstrate the efficacy of general behavioral principles of ABA for behavior change in a variety of populations. (New York State Department of Health, 1999)
4. There are studies that address some of the concerns raised by the committee: e.g., better outcomes with more hours of intervention (Lovaas, 1987), equal outcomes for parent-led vs. agency-led intervention (Sallows & Graupner, 2005). However, the authors exclude them from the AHRQ report without explaining why.

5. The AHRQ report arbitrarily holds psychosocial interventions to a much higher standard than medication.
 - a. If one is to take this report at face value, it appears there are no interventions for autism that should be used -- aside from medication, despite the fact that medication has no effect on some of the more debilitating symptoms of social and adaptive skill development.
6. Of course RCT's are gold standard for intervention outcome studies. However, the authors fail to acknowledge that this type of research is extremely difficult, if not impossible, and perhaps ethically inappropriate, to implement with an intervention that is done intensively over 2 years of the individual's life.
 - a. There is enough evidence available to suggest that intensive and comprehensive behavioral intervention is an effective treatment for individuals with autism. Therefore there are serious ethical considerations involved in completing this type of research, specifically in terms of random assignment to groups, as control group individuals will then be denied effective treatment for the entire length of the study.
 - b. Additionally, these types of interventions require a great deal of time in order to determine efficacy (unlike medication trials in which effects may manifest within months), so such studies need to be carried out over a lengthier period of time.
 - c. While I understand the need for standardization of treatment, one of the hallmarks of behavioral intervention for individuals with autism is individualization. In order to be effective, treatment needs to be individualized, and therefore treatment fidelity scores may be lower if certain individuals require greater amounts of individualization.
 - d. Finally, creating a comparison group which is truly indistinguishable from the treatment group is excessively difficult given that treatment is 40 hours per week of intensive intervention adhering to a specific treatment protocol. Creating a comparison group that replicates that in a way that participants are truly blind to the condition they are assigned to is extremely challenging.
7. The reviewers are overly critical in the analysis of the literature (e.g., diagnostic standards are criticized along with the use of disparate outcome measures) without taking into account the validity of the measures or diagnostic procedures used.
8. Criticizing the behavioral literature for "the duration of treatment and follow-up being relatively short" is confusing. In the study done by Lovaas in 1987 treatment took place over 2 years with a follow-up 7 years later. There have also been several other studies done that have been several years in length (Howard et al., 2005; Eikeseth et al., 2002; Eikeseth et al., 2007 for example). Given that medication studies take place over several weeks with follow-up less than six months later, again this seems like an unfair and willfully arbitrary criticism of the behavioral literature.
9. Nowhere in the report does the review panel directly address that ABA is currently the treatment with the largest amount of research to back it done to date. While there are admittedly weaknesses in this body of literature, it is one of the most extensively studied interventions for individuals with autism and the results are better and more comprehensive than any other intervention.

In summary, the AHRQ report is inconsistent and arbitrary in its recommendations of intervention techniques for individuals with autism. Based on these recommendations the only available and funded treatments would be two medications, which have a relatively limited database of support in the literature, and which only target selected aspects of the overall deficits of individuals with autism. Again, while the shortcomings of the existing literature base are acknowledged, ABA is currently the most comprehensively researched intervention and to date the most effective for all deficit areas in individuals with autism.

The Healthcare Technology Agency report: Difficult to interpret and incomplete

1. The HTA report was difficult to interpret and incomplete. There was a lack of information on the ratings assigned to the studies under review, rendering such ratings difficult, if not impossible, to interpret. Additionally, several reports completed by other state or federal agencies were inexplicably omitted from the HTA review. The following reports were omitted from the review (annotations are mine):

2. 1. Maine Department of Health and Human Services & The Maine Department of Education (2009)
 - a. Early Intensive Behavioral Intervention – *Established evidence*
 - b. Applied Behavior Analysis for Challenging Behavior – *Established evidence*
 - c. Applied Behavior Analysis for Communication – *Established evidence*
 - d. Applied Behavior Analysis for Social Skills – *Established evidence*
3. New York State Department of Health (1999)
 - a. Principles of ABA and behavior intervention strategies should be included as an important element of any intervention program for young children with autism – *Strong evidence*
 - b. Intensive behavioral programs include as a minimum approximately 20 hours per week of individualized behavioral intervention using ABA techniques (not including time spent by parents) – *Strong evidence*
 - c. Precise number of hours of behavioral intervention vary depending on a variety of child and family characteristics. Considerations in determining the frequency and intensity of intervention include age, severity of autistic symptoms, rate of progress, other health considerations, tolerance of the child for the intervention and family participation – *Strong evidence*
 - d. Effective interventions based on ABA techniques used between 18 and 40 hours per week of intensive behavioral intervention by a therapist trained in this method – *Strong evidence*
4. American Academy of Pediatrics (2007) – Management of Autism Spectrum Disorders
 - a. ‘The effectiveness of ABA-based intervention in ASD’s has been well documented through 5 decades of research by using single-subject methodology and in controlled studies of comprehensive early intensive behavioral intervention programs in university and community settings. Children who receive early intensive behavioral treatment have been shown to make substantial, sustained gains in IQ, language, academic performance, and adaptive behavior as well as some measures of social behavior, and their outcomes have been significantly better than those of children in control groups.’
5. Department of Defense Report and Plan on Services to Military Dependent Children with Autism (2007)
 - a. ‘Applied behavior analysis (ABA), a systematized process of collecting data on a child’s behaviors and using a variety of behavioral conditioning techniques to teach and reinforce desired behaviors while extinguishing harmful or undesired behaviors, is one of the best studied interventions. Time-limited, focused ABA methods have been shown to reduce or eliminate specific program behaviors and teach new skills to individuals with autism.’ Page 4
 - b. ‘A large body of research has demonstrated substantial progress in response to specific intervention techniques in relatively short periods of time (e.g., several months) in many specific areas, including social skills, language acquisition, nonverbal communication, and reductions in challenging behaviors. Longitudinal studies over longer periods of time have documents changes in IQ scores and in core deficits (e.g., joint attention), in some cases related to treatment, that are predictive of longer term outcomes. However, children’s outcomes are variable, with some children making substantial progress and others showing very slow gains.’ Page 7
6. US Surgeon General (1999)
 - a. ‘Thirty years of research demonstrated the efficacy of applied behavioral methods in reducing inappropriate behavior and in increasing communication, learning, and appropriate social behavior.’
7. National Institute of Mental Health - <http://www.nimh.nih.gov/health/publications/autism/complete-index.shtml>
 - a. ‘Among the many methods available for treatment and education of people with autism, applied behavior analysis (ABA) has become widely accepted as an effective treatment. *Mental Health: A Report of the Surgeon General* states, “Thirty years of research demonstrated the efficacy of applied behavioral methods in reducing inappropriate behavior and in increasing communication, learning, and appropriate social behavior”’. The basic research done by Ivar Lovaas and his colleagues at the University of California, Los Angeles, calling for an intensive, one-on-one child-teacher interaction for 40 hours a

week, laid a foundation for other educators and researchers in the search for further effective early interventions to help those with ASD attain their potential.'

8. National Research Council (2001) – Educating Children with Autism
 - a. 'There is general agreement across comprehensive intervention programs about a number of features of effective programs. However, practical and, sometimes, ethical considerations have made well-controlled studies with random assignment (e.g., studies of treatments that systematically vary only one dimension) almost impossible to conduct.' Page 6

In summary, the HTA report is difficult to interpret, given the lack of clarity about how selected studies were rated, and, more bewilderingly, incomplete, given the inexplicable omission of critical reports, authored by highly reputable institutions – among them the NIMH, the United States Surgeon General, and the American Academy of Pediatrics.

Understanding Efficacious Treatment for Children with Autism

It should be noted that within Washington State there are agencies that support the use of ABA for individuals with developmental disabilities. For example in the Children' Intensive In-Home Behavior Support Services (CIIBS) program, the primary modality of treatment is Positive Behavioral Support Model, which is one branch of ABA. Children with autism can access these services, and thus the state is already funding ABA for children with autism at some level.

Finally and most importantly, cost-benefit analyses of treatment interventions are founded on the evaluation of fiscal benefit of early and intensive behavioral intervention with individuals with autism. While not every individual will be a best outcome case, there are other benefits to intensive behavioral intervention (e.g., increased functional vocabulary, increased self-help skills, decreases in problematic behaviors, etc.). Thus, even if an individual does not respond optimally to intervention, there are lifetime benefits to intervention, which result in lower levels of care throughout the individual's adult life. Estimates vary, but the conservative estimate on lifetime savings per individual is \$850,000 to \$1,200,000 (Jacobson, Mulick, & Green, 1998). In a report by Columbia Pacific Consulting firm, in an affidavit to Douglas G. Hildebrand, the authors report that even in the lowest success group savings are likely to amount to \$642,200 (individuals with better outcomes are associated with cost savings up to \$1,368,900). With the increasing prevalence of autism, early intervention has the potential to save the government – and by extension, all taxpayers – a significantly massive amount of money across the lifespan of an individual with autism.

In summary, ABA intervention is a well researched and well-established intervention for individuals with autism.

While – as across many domains of child mental health -- there is still a significant amount of research that remains to be done, the reports submitted as information for this committee's decision would suggest no intervention aside from two medications should be funded in the treatment of children with autism. If that is the case, why do we send children with autism to school? It is a cynical and false argument to claim there is no effective form of intervention that is worth spending taxpayers' money on. We might as well revert back to simply institutionalizing individuals with autism shortly after they are born, if we truly believe there is no hope of their either learning more adaptive behavior or of learning to control their problematic behavior, aside from long-term use of medication with some relatively serious side effects.

By contrast, I would argue that the data shows that we can teach individuals on the spectrum many skills and decrease problematic behaviors using the principles of ABA. These strategies and techniques not only have the short-term benefit of increasing desirable behaviors and decreasing problematic behaviors, they also have the long-term benefit of decreasing the level of care an individual will require throughout their life span, thus saving taxpayers a significant amount of money.

The decision the Committee makes will have profound, significant and lasting impact on not only the lives of individuals affected by autism, but also on the taxpayers of this state. A scenario in which children are denied efficacious treatment, and taxpayers are burdened with the care of untreated adults is both tragic and wasteful.

It is critical that a decision of this magnitude and significance be made in a manner that is transparent, reasoned, credible and evidence-based.

I respectfully suggest that the AHRQ and HTA reports do not meet this standard, and as such, are an inappropriate foundation for decision-making.

I hope you will consider the points made in this letter in making your decision.

Yours truly,
Sara White, PhD, BCBA-D
Psychologist

Co-signatory:
James Harle, MD
Child and Adolescent Psychiatrist

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ABA therapy is very effective & helpful for Autistic children. I wish that my kid can have it but it's not covered by our insurances.

Tam Dang

Using Participant Data to Extend the Evidence Base for Intensive Behavioral Intervention for Children With Autism

Sigmund Eldevik

Akershus University College (Lillestrom, Norway)

Richard P. Hastings and J. Carl Hughes

Bangor University (Bangor, Wales)

Erik Jahr

Akershus University Hospital (Lorenskog, Norway)

Svein Eikeseth

Akershus University College

Scott Cross

Lovaas Institute for Early Intervention (Culver City, CA)

Abstract

We gathered individual participant data from 16 group design studies on behavioral intervention for children with autism. In these studies, 309 children received behavioral intervention, 39 received comparison interventions, and 105 were in a control group. More children who underwent behavioral intervention achieved reliable change in IQ (29.8%) compared with 2.6% and 8.7% for comparison and control groups, respectively, and reliable change in adaptive behavior was achieved for 20.6% versus 5.7% and 5.1%, respectively. These results equated to a number needed to treat of 5 for IQ and 7 for adaptive behavior and absolute risk reduction of 23% and 16%, respectively. Within the behavioral intervention sample, IQ and adaptive behavior at intake predicted gains in adaptive behavior. Intensity of intervention predicted gains in both IQ and adaptive behavior.

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There is a growing body of evidence that intensive behavioral intervention can result in significant improvement in the intellectual, social, adaptive, and language functioning of young children with autism spectrum disorders (Cohen, Amerine-Dickens, & Smith, 2006; Eikeseth, Smith, Jahr, & Eldevik, 2007; Hayward, Eikeseth, Gale, & Morgan, 2009; Howard, Sparkman, Cohen, Green, & Stanislaw, 2005; Lovaas, 1987; Remington et al., 2007; Sallows & Graupner,

2005; Smith, Groen, & Wynn, 2000). A sizeable minority of children might even reach the average to superior range within one or more of these areas of functioning following intervention (Cohen et al., 2006; Eikeseth et al., 2007; Hayward et al., 2009; Howard et al., 2005; Lovaas, 1987; Remington et al., 2007; Sallows & Graupner, 2005; Smith et al., 2000). There is also promising, although limited, evidence that these outcomes may maintain over the long term into adolescence

following the cessation of intervention (McEachin, Smith, & Lovaas, 1993). Such data have led to positive conclusions about the evidence base for intensive behavioral intervention in two recent narrative reviews (Eikeseth, 2009; Rogers & Vismara, 2008). According to Rogers and Vismara, clinic-based intensive behavioral intervention (or what they call the Lovaas treatment approach) can be considered well-established based on formal criteria (Chambless et al., 1996, 1998; Chambless & Hollon, 1998).

Although there are statistically significant group differences in controlled studies, a more thorough analysis of what the results mean in clinical terms is also required. Such an analysis can be done in several ways. One approach is to examine outcome using meta-analysis of aggregated data that are typically reported in published studies, such as the mean pre- and posttest scores in the experimental and control groups (e.g., Borenstein, Hedges, Higgins, & Rothstein, 2009; Cooper, Hedges, & Valentine, 2009). Reichow and Wolery (2009) recently conducted a synthesis of the research, including an aggregated data meta-analysis on the effects of intensive behavioral intervention for children with autism. Based on 12 studies, they found a weighted mean change (i.e., pre-post change in intervention groups only) effect size for IQ of .69 following intensive behavioral intervention. Such an effect size would normally be considered clinically meaningful. In a second aggregated data meta-analysis of 9 controlled studies of intensive behavioral intervention, using a weighted mean difference effect size, Eldevik et al. (2009) found a large effect for IQ change in favor of intensive behavioral intervention, Hedges' $g = 1.10$, 95% CI = .87, 1.34, and a smaller, although still statistically significant, effect for change in adaptive behavior composite (ABC) scores, Hedges' $g = .66$, 95% CI = .41, .90.

An especially significant feature of the Eldevik et al., (2009) analysis is that individual participant data were obtained from the authors of studies selected for the review. Thus, the aggregated data meta-analysis was based on individual study effect sizes calculated using the same method, for similar evaluation periods, and following the removal of children whose data appeared in more than one report. An aggregated data meta-analysis of individual study effect sizes derived from individual participant data is a recommended first step in any analysis of

evidence for an intervention using individual level data (Cooper & Patall, 2009). A second step is to conduct an individual participant data meta-analysis proper. Such an analysis is likely to have important benefits over aggregated data meta-analysis, including the possibility of dividing the individual participants into new subgroups and applying different statistical methods (Cooper & Patall, 2009). This form of meta-analysis (sometimes also called *mega-analysis*) involves the combination of data across studies into a single intervention and comparison/control group(s).

Given that the outcome for individual children in intensive behavioral intervention studies varies considerably (Howlin, Magiati, & Charman, 2009), an important step when examining the evidence base for this intervention is to evaluate meaningful changes at the level of individual children. To date, the method for assessing which children achieve meaningful change (best outcome) has not been consistent in existing research. Lovaas (1987) defined *best outcome* as intellectual functioning (IQ) scores within the normal range and successful first grade performance in public schools. Sallows and Graupner (2005) used the terms *rapid learners* and *moderate learners* to define similar outcomes. A more objective method for establishing meaningful change at the level of the individual child is needed.

Remington et al. (2007) used the Reliable Change Index (N. Jacobson & Truax, 1991), a construct borrowed from psychotherapy outcome research, to examine meaningful change in their intensive behavioral intervention controlled study. *Reliable change* is the amount by which an outcome measure needs to change before one can be 95% certain that the change cannot be accounted for by the variability of scores in the sample and/or measurement error. The reliable change index is computed by subtracting the pretest scores from the posttest scores and then dividing by the standard error of difference. The standard error of difference is, in turn, computed directly from the standard error of measurement and describes the distribution of change scores that would be expected if no change occurred (N. Jacobson & Truax, 1991, p. 14).

Using N. Jacobson and Truax's formula, Remington et al. (2007) found that 6 out of 23 children (26%) in their intensive behavioral intervention group achieved positive reliable change in IQ after 2 years, whereas 3 out of 21

(14%) in the treatment as usual group achieved this level of change, and the IQs of 3 children in this group also decreased to a reliable extent. To date, no other published intensive behavioral intervention study has used this objective criterion to identify best outcome children, and Remington et al. only reported this analysis for IQ and not other domains of outcome.

One advantage of establishing a dichotomous outcome variable for change in intensive behavioral intervention at the level of individual participants (i.e., achieved reliable change or not) is that effect size statistics commonly used to evaluate the potency of health interventions can be generated. Such statistics include the number needed to treat and absolute risk reduction (Straus & Sackett, 2005). These statistics are particularly helpful as simple ways to communicate information about interventions to policymakers. The number needed to treat represents the number of children who would need to be treated with a specified intervention to obtain one additional success over the success rate in a comparison intervention. For example, number needed to treat = 4 means that for every four children who are treated with intervention X, one additional child will respond to this intervention who would not have responded to a comparison intervention. A result of number needed to treat = 1 means that all children receiving an intervention succeed when they would not have done so following a comparison intervention. In other words, the larger the number needed to treat, the less effective the treatment relative to the comparison (Kraemer et al., 2003).

Absolute risk reduction is computed in a similar way as number needed to treat but expressed as a measure of the difference in percentage response between two interventions (Pinson & Gray, 2003). When the absolute risk reduction is used as a measure of intervention effectiveness, the results are usually given in negative outcome. This means that an effective intervention will reduce negative outcome or, put another way, reduce the risk of having bad outcome. For example, if in intervention A, 50% of patients do not respond to intervention and in intervention B, 90% do not respond to intervention, the absolute risk reduction (also called risk difference) is 40% in favor of intervention A.

A further advantage of establishing an objective criterion for meaningful outcome for individual children with autism receiving intensive

behavioral intervention is that the search for correlates or predictors of intensive behavioral intervention outcome can become more consistent. For example, the 6 children who achieved reliable change following intensive behavioral intervention in the Remington et al. (2007) study were compared with the 3 children in the intensive behavioral intervention group whose IQs decreased. The children who met reliable change criteria had higher IQ, mental age (MA), Vineland Adaptive Behavior Scales–VABS (Sparrow, Balla, & Cicchetti, 1984) Composite scores, along with higher VABS Communication and Socialization scores at intake. In addition, these best outcome children at intake had lower VABS Motor scores, more behavior problems on the Developmental Behavior Checklist (Einfeld & Tonge, 1995), and more autistic symptoms on the Developmental Behavior Checklist autism algorithm (Einfeld & Tonge, 2002), but also had fewer treatment hours in their second year of intensive behavioral intervention.

Apart from the Lovaas (1987) intensity comparison (40 vs. ≤ 10 hr), intensive behavioral intervention studies have not been explicitly designed to explore moderators of outcome. Rather, as in the Remington et al. (2007) study, various methods to examine correlates of outcome have been adopted. Correlates of outcome explored in existing research include rates of learning early in intervention or initial skill acquisition (Sallows & Graupner, 2005; Weiss, 1999), age at intake (Harris & Handleman, 2000), IQ at intake (Ben-Itzhak & Zachor, 2007; Harris & Handleman, 2000), initial social skills (Ben-Itzhak & Zachor, 2007; Eikeseth, Smith, Jahr, & Eldevik, 2007), toy play and socially avoidant behavior at intake (Sherer & Schreibman, 2005), and autism subtype (Beglinger & Smith, 2005). Notably, despite its potential significance to the intensive behavioral intervention debate, the intensity of intervention has been shown to relate to outcomes only in Lovaas' (1987) original experimental comparison. However, most salient in the current context is that given there is no consistency in the definition of *meaningful outcome* in intensive behavioral intervention, there is currently no evidence base that can be used to identify children at intake who are likely to achieve best outcome, let alone to prescribe a certain intensity (or duration) of intervention.

In the present study we collected individual participant data by contacting authors and from

published intensive behavioral intervention outcome studies identified via a systematic review. We then used all of these data to establish whether each child met reliable change criteria for changes in IQ or adaptive behavior after approximately 2 years of intervention. These data were then used to address two aims. First, we conducted an individual participant data meta-analysis of intensive behavioral intervention outcomes against those of control/comparison interventions. This extended the work of Eldevik et al. (2009) and Reichow and Wolery (2009) because both controlled and uncontrolled studies could be included in the analysis, the data were at a different level of analysis than these authors' aggregated data meta-analyses, and effect size statistics based on dichotomous outcomes were adopted. Our second aim was to explore predictors of outcome in children who had received intensive behavioral intervention. Using this analysis we were able to extend beyond the small *n* analyses from individual published studies and to facilitate a more sophisticated analysis of outcome prediction in one important respect. We were able to explore both main effects as well as interactions between key variables (e.g., age at intake combined with IQ at intake) as potential predictors. Such analyses were not possible in previous research because participant numbers were too small.

Method

Searching Strategy and Data Collection

We conducted a comprehensive literature search using PsycINFO, Pubmed, and ERIC databases (up to March 2008) using a combination of the following terms: *behavior analytic, behavioral, early, intervention, and autism* and/or *pervasive developmental disorder -not otherwise specified* (PDD-NOS). The first author read the titles and abstracts of all papers collected from this initial search; studies that contained standardized outcome data on the effects of behavioral intervention for young children with autism were obtained for more detailed coding. The first author manually browsed the reference section of each study in an attempt to locate other studies that might have been missed during the electronic search.

Following this selection process, we developed a coding scheme (available from the first author) and coded the selected studies in two main ways. First, we coded whether the children

had received behavioral intervention that adhered to the common elements described by Green, Brennan, and Fein (2002, p. 70); that is, (a) intervention was individualized and comprehensive, addressing all skill domains; (b) many behavior analytic procedures were used to build new repertoires and reduce interfering behavior (e.g., differential reinforcement, prompting, discrete-trial instruction, incidental teaching, activity-embedded trials, task analysis, and others); (c) one or more individuals with advanced training in applied behavior analysis and experience with young children who had autism directed the intervention; (d) typical developmental sequences guided selection of intervention goals and short-term objectives; (e) parents served as active cotherapists for their children; (f) intervention was delivered in one-to-one fashion initially, with gradual transitions to small- and large-group formats when warranted; (g) intervention typically began in the home and was carried over into other environments (e.g., community settings), with gradual, systematic transitions to preschool, kindergarten, and elementary school classrooms when children developed the skills required to learn in those settings; (h) programming was intensive, including 20 to 30 hr of structured sessions per week plus informal instruction and practice throughout most of the children's other waking hours, year round; (i) in most cases, the duration of intervention was 2 or more years; and (j) most children started intervention in the preschool years, when they were 3 to 4 years of age.

The second way we coded the selected studies was by applying a series of true/false scores using the following criteria: (a) the participants were, on average, between 2 and 7 years old when intervention started; (b) the children were independently diagnosed with autism or PDD-NOS; (c) a full-scale measure of intelligence and/or a standardized measure of adaptive behavior, such as the VABS, was conducted at intake and after intervention—we excluded studies in which the researchers had primarily administered a nonverbal intelligence measure, such as the Leiter International Performance Scale-Revised (Roid & Miller, 1997) or the Merrill-Palmer Scale of Mental Tests (Stutsman, 1948) because the results of such assessments may differ substantially from those of full scale intelligence tests (Scheuffgen, Happe, Anderson, & Frith, 2000); (d) the duration of intervention was between 12 and 36 months; (e) the study was not a case study (or series of case

studies); and (f) the results had been published in a peer-reviewed journal. In addition, if data on control or comparison groups were reported, these were included and grouped according to the criteria given below. If all the above criteria were met, the authors of the study were approached and asked to provide data on individual children, if this was not already available in the published paper.

Data on other groups included in intensive behavioral intervention evaluation studies were coded as either comparison group data, which meant that another form or forms of intervention of similar intensity (in terms of 1:1 hours) was specified, or control group data, which meant that no or a considerably less intensive alternative intervention was specified, often merely described as “treatment as usual.” Although it would probably be impossible to determine whether the children in the comparison groups had a specific common provision (even within a single study), classifying the studies in this way could yield useful information. For example, it is important to establish whether intensive behavioral intervention might be efficacious when compared to other similarly intensive interventions or only when compared against an ill-defined treatment as usual.

The initial electronic and manual searches resulted in 2,150 potential hits in total across the databases. Through the screening process, we selected 33 papers for closer examination and detailed coding. We also chose one of the database searches that had resulted in 607 potential hits for a reliability check. The screening results from the first author were compared to those of a second coder (another author) using the same decision criteria. Agreement was high overall in terms of whether to select a paper for further coding, Cohen’s $Kappa = .85$. Notably, disagreements only occurred because the second screener included fewer studies than did the first author. Thus, there were no instances of the second screener including a study for further coding that was not already included by the first author.

The remaining 33 studies were then coded by the first author and two independent scorers (master’s level students in behavior analysis) using the true/false criteria described above. Agreement was calculated between the first author and each of the independent scorers separately by dividing the total number of agreements by the total number of agreements plus disagreements and

multiplying by 100. Initial agreement was high in both cases (91% and 94%, respectively), and the few disagreements that occurred were resolved after brief discussions. We excluded 18 out of the 33 studies for one or more of the following reasons: (a) 7 had inadequate intake and/or outcome data, most often reporting primarily Performance IQ instead of Full Scale IQ (Bibby, Eikeseth, Martin, Mudford, & Reeves, 2002; Drew et al., 2002; Fenske, Zalenski, Krantz, & McClannahan, 1985; Luiselli, Cannon, Ellis, & Sisson, 2000; Magiati, Charman, & Howlin, 2007; Sheinkopf & Siegel, 1998; Solomon, Necheles, Ferch, & Bruckman, 2007); (b) in 5 of the studies, the duration of intervention was too short to meet inclusion criteria (Harris, Handleman, Gordon, Kristoff, & Fuentes, 1991; Ingersoll, Schreibman, & Stahmer, 2001; Reed, Osborne, & Corness, 2007a, 2007b; Stahmer & Ingersoll, 2004); (c) in 2 papers the researchers reported data from case studies only (Butter, Mulick, & Metz, 2006; Green et al., 2002); (d) in 3 of the studies, investigators reported data that were already included in other studies (Beglinger & Smith, 2005; Eikeseth et al., 2007; McEachin et al., 1993); and (e) upon closer scrutiny one of the studies provided intervention that did not meet the definition of behavioral intervention (Gabriels, Hill, Pierce, Rogers, & Wehner, 2001).

In only 4 of the 15 remaining studies did researchers report individual outcome data in the original published paper. The authors of the 11 remaining studies were contacted and asked to provide data on individual children; all of them agreed. However, individual data from Control Group 2 ($n = 21$) in the Lovaas (1987) study were not available. Furthermore, data from 4 children in the comparison group of one study (Eldevik, Eikeseth, Jahr, & Smith, 2006) were extracted because they were also in the comparison group of another study included in the analysis (Eikeseth, Smith, Jahr, & Eldevik, 2002). One of the authors whom we contacted also volunteered an additional study (Hayward et al., 2009); because this study had been subject to peer review and met all other criteria, it was also included in the present analysis. Figure 1 presents a flowchart of the search and selection procedure.

Table 1 summarizes the main characteristics of the studies included in this analysis, the mean age of participants at intake, and their mean IQ and adaptive behavior scores at intake and postintervention. Furthermore, the mean intensi-

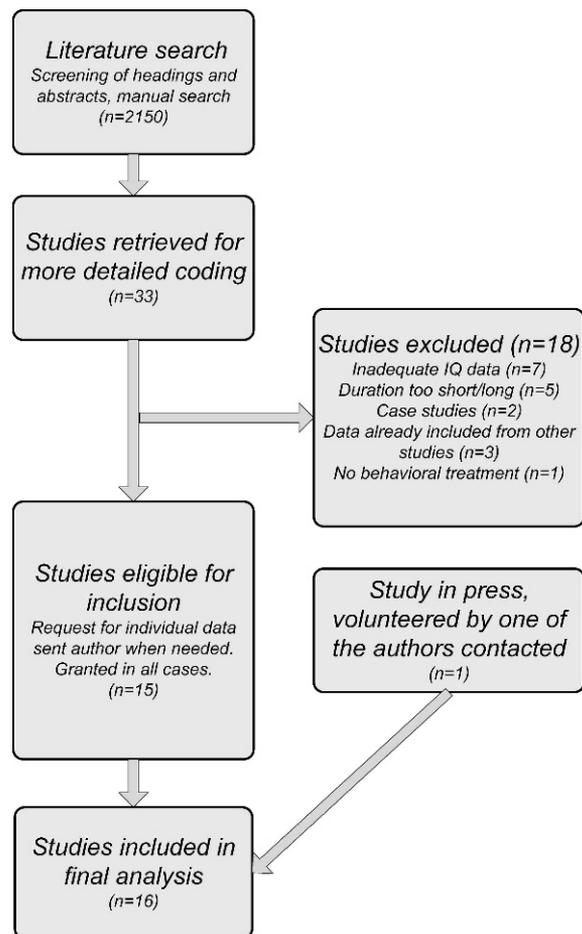


Figure 1. Flowchart on the procedure for selecting studies.

ty in terms of weekly hours and duration is provided, and the research design and assignment procedures employed are briefly described along with any inclusion criteria employed in the original paper. If the researchers reported outcome data at more than one point in time, we chose the point that was closest to a 2-year duration of intervention.

Participants

Individual data were available for 453 participants, including 309 who had received intensive behavioral intervention, 105 in control groups, and 39 in comparison groups. Due mainly to different assessment protocols (i.e., because the measures were not included in the research), some IQ data (1 study) and adaptive behavior data (2 studies) are missing (see below). A one-way ANOVA showed that the three groups were similar on intelligence measures at intake. Chil-

dren in the comparison intervention group were older than those in the other two groups at intake, and children in the control group had higher VABS Composite scores (see Table 2). However, the total sample, as well as the separate subgroups with the sample, are generally representative of the autism population (Volkmar & Klin, 2005). Because not all authors were able to provide data on the gender of each child (38.5% missing), these data were not included in the present analysis. Within the intensive behavioral intervention group, the number of weekly intervention hours for each child was only available for 75 out of 309 children (24.3%). To include intensity as a variable, we decided to create a median split of the overall data using a hierarchy of evidence. First, we used the data provided by the author on intervention intensity for each child if these data were available. Second, we used the mean weekly hours of intervention for the intensive behavioral intervention studies were typically based on reports that all children had been exposed to at least the relevant number of weekly hours specified in the intervention. In total, 152 children (49.5%) received 36 or more hours of intervention on a weekly basis, and 155 children (50.5%) received fewer than 36 hr of weekly intervention.

Child Measures

Intelligence. The Bayley Scales of Infant Development (BSID), either the first or second edition (Bayley, 1969, 1993), were most often used for the youngest children or the children who scored below the basal on other intelligence tests. The BSID is a measure of mental developmental level for children up to 42 months. It yields a Mental Developmental Index, which is considered broadly equivalent to an IQ. For the older and higher functioning children, the most frequently used measures of intelligence were the Stanford-Binet Intelligence Scale: Fourth Edition (Thorndike, Hagen, & Sattler, 1986), the Wechsler Preschool and Primary Scale Intelligence-Revised (Wechsler, 1989), the Wechsler Intelligence Scale for Children-Revised (Wechsler, 1974), or the Wechsler Intelligence Scales for Children-Third Edition (Wechsler, 1993). All of these tests have been validated and used extensively for children with developmental delays and autism (Newsom

& Hovanitz, 1997). If the child scored below the norms on a test, researchers generally computed a ratio IQ by dividing the obtained MA with chronological age and multiplying by 100. Unfortunately, we did not have data regarding which tests were used for each child at what point nor information on whom a ratio IQ was used. IQ outcome data were obtained from a total of 422 children (31 missing). These were divided as follows: 279 children in the intensive behavioral intervention groups (30 missing), 104 children in the control groups (1 missing), and 39 children in the comparison groups (0 missing).

Adaptive behavior. The VABS, which was the measure for adaptive skills in all studies included in this research, provides standard scores for communication, daily living skills, and socialization; and for children under 6 years old, motor skills. It also yields a total ABC. In the present study we only used the ABC scores because we did not have access to the various domain scores. The VABS is widely regarded as the best interview for assessing adaptive levels for children with autism (Klin, Saulnier, Tsatsanis, & Volkmar, 2005). Data on adaptive behaviors were obtained from a total of 357 children (96 missing): 248 children in the intensive behavioral intervention groups (61 missing), 70 children in the control groups (35 missing), and 39 children in the comparison groups (0 missing).

Data Analysis Procedure

To evaluate effectiveness of behavioral intervention at the level of individual children, we applied the statistical approach outlined by N. Jacobson and Truax (1991). The formula for computing reliable change requires that one is able to determine the stability and distribution of the test scores (in this case IQ and ABC scores). Because neither of these are well-established for young children with autism, we decided to use our relatively large sample to generate suitable information (following Remington et al., 2007). We estimated the stability of test scores over 2 years by finding the correlation between pre- and postscores in the control group, where no identified intervention had been applied and, thus, where stability might be better estimated than from groups receiving active interventions. We used intake data to calculate the *SD* for test scores from the whole sample of 453 children. Using the formula reported in N. Jacobson and

Truax (1991, p. 14), we established the absolute change in scores required to achieve a reliable change index score of 1.96 (95% certainty).

In some intensive behavioral intervention studies, investigators excluded children with intake IQs at or below 35 (Cohen et al., 2006; Sallows & Graupner, 2005; Smith et al., 2000). Given this practice, we conducted analyses on the whole sample and also repeated them for the sample ($n = 387$) whose intake IQs were 35 or above. Thus, we calculated change scores above which reliable change was indicated for the whole sample and for the 35+ IQ sample. To be considered reliable, the change in IQ had to be at least 27.4 points, rounded to 27 for the purposes of this analysis (26.6 for the subset of children with IQ > 35 at intake); for the ABC the change had to be at least 21.0 points (21.3 for the subset of children with IQ > 35 at intake). The more lenient criterion on the VABS mainly reflected a smaller *SD* in the test scores at intake. None of the analyses reported here revealed a different pattern of results when the children with intake IQs below 35 were excluded; thus, no further results excluding those children are reported.

After classifying each child in terms of whether his or her intellectual functioning and adaptive levels changed to a reliable extent, we computed number needed to treat and absolute risk reduction (Laupacis, Sackett, & Roberts, 1988). This was done for the total sample (i.e., an individual participant data meta-analysis) and, when possible, for the individual studies (i.e., studies that had a control or comparison group). The latter were included to illustrate the degree of variability across studies. To conduct the number needed to treat and absolute risk reduction calculations, we used readily available free access online calculators (Straus, Newton, & Tomlinson, 2004).

To explore predictors of intensive behavioral intervention outcomes, we conducted a multiple regression analyses for the behavioral intervention group ($n = 309$). The dependent variables were absolute change scores for IQ and ABC. We used absolute change scores rather than a dichotomous outcome variable for ease of analysis and to ensure the maximum possible variability in the dependent variable given the difficulties inherent in searching for moderated effects in multiple regression analysis (McClelland & Judd, 1993). The variables we investigated as possible predictors were age at intake, IQ at intake, ABC at

Table 1. Characteristics of the Studies Included in the Present Analyses

Country/Study/Group	Age		Pretest				Posttest			
			IQ		ABC		IQ		ABC	
	Mean	SD	Mean	SD	Mean	SD	Mean	SD	Mean	SD
United States										
Lovaas (1987)										
IBA ^c (clinic)	34.6	8.9	62.9	13.7	–	–	83.3	28.6	–	–
Control	40.9	10.3	57.1	14.5	–	–	50.1	22.4	–	–
Anderson et al. (1987)										
IBI (clinic)	42.8	11.8	55.0	18.9	47.7	8.2	60.6	25.1	57.7	15.3
Smith et al. (1997)										
IBI (clinic)	36.0	6.9	27.8	4.9	50.3	9.1	35.8	14.3	51.7	17.9
Control	38.0	5.4	27.3	5.4	–	–	24.0	8.2	–	–
Weiss (1999)										
IBI (parent)	42.0	–	–	49.9	7.8	–	–	83.6	28.3	–
Harris & Handleman (2000)										
IBI (clinic)	49.0	8.8	59.3	24.2	–	–	77.6	28.6	–	–

(Table 1 continued)

Table 1. Extended

Intensity		<i>n</i>	Gender		Design/Assignment/ Inclusion ^a	Comments ^b
Hr	Mon.		M	F		
40 <10	24–36 24	19 19	16 11	3 8	QCT/staff availability and archives Included if CA < 40 months if mute or CA < 46 months if echolaic and prorated MA of > 11 months at CA 30 months	Five subjects deemed untestable at intake, 3 in experimental group and 2 in Control Group 1 Intelligence scores based on MA score from Vineland Social Maturity Scale (Doll, 1953) were used in these cases INDA ^c
20	12–24	14	11	3	UCT/parent willingness and geographical Included if CA < 72 months	Ratio IQ and VABS computed on basis of tables in original paper Intensity set to 20 but was reported to be flexible between 15 and 25 hr per week One child was 18 months and 1 child was 23 months at intake Duration was either 1 or 2 years Postmeasures conducted 3– 4 years after treatment in some cases VABS data available only for 6 of 11 children in the IBI group Control group received minimal treatment
30 <10	24 24	11 10	11 8	0 2	QCT/archival data Included if CA ≤ 46 months and IQ < 35	Workshops were done every 4– 6 weeks Mix of clinic and parent managed programs Entire caseload of clinician from 80 children enrolled at center No. of hr per week between 35 and 45 Follow-up testing done 4–6 years after treatment Duration of treatment 1, 2, or 3 years INDA
40	24	20	19	1	UCT/enrollment center	
40	12–36	27	23	4	UCT/enrollment center	

Table 1. Continued

Country/Study/Group	Age		Pretest				Posttest				
			IQ		ABC		IQ		ABC		
	Mean	SD	Mean	SD	Mean	SD	Mean	SD	Mean	SD	
Smith et al. (2000)											
IBI (clinic)	36.1	6.0	50.5	11.2	63.7	9.6	66.5	24.1	61.3	28.7	
Control	35.7	5.4	50.7	13.9	65.2	9.0	50.5	20.4	59.9	16.7	
Smith et al. (2000)											
IBI (parent)	35.8	4.8		11.8	54.8	4.5	58.3	19.3	60.2	16.7	
Sallows & Graupner (2005)											
IBI (clinic)	33.7	3.9	48.8	8.8	59.8	5.7	70.8	24.6	63.4	23.6	
IBI (parent)	30.2	3.9	44.4	8.2	54.4	5.3	63.8	23.5	58.9	21.8	

(Table 1 continued)

Table 1. Extended Continued

Intensity		<i>n</i>	Gender		Design/Assignment/ Inclusion ^a	Comments ^b
Hr	Mon.		M	F		
24.5 <10	24 ~24	15 13	12 11	3 2	RCT/matched-pair random Included if CA < 42 months and ratio IQ between 35 and 75	Clinic directed group: no. of hr for IBI group for first year in treatment Gradual reductions in Year 2 Treatment phased out after 18 months for children responding slowly Average duration 33 months Parent managed group: 5 hr a week of parent training for first 3–9 months, parents asked to do 5 hr a week in between sessions: total < 10 hr per week of ABA + 12.5 hr of special education classes per week ABA treatment hr second year presumed to be gradually decreasing, school hr presumed to be the same Follow-up testing at CA 7–8 years Duration between testing on average 54 months Autism and PDD-NOS lumped together in the present analysis INDA
26	24	6	6	0	UCT/Consecutive referrals Included if CA < 48 months	Two boys deemed untestable and IQ set to 30 Posttreatment after 2–3 years Children had to be under 48 months at intake Average of 26.2 hr a week the first 5 months, after that 30 hr for 5 of the children (1 dropped out) Supervision monthly
38 31	24 24	13 11	11 8	2 3	RCT/matched-pair random Included if CA < 42 months and ratio IQ ≥ 35	In order to keep as many variables as possible constant, Year 2 outcome data were obtained from the authors Intensity data from Sallows & Graupner (2005) INDA

Table 1. Continued

Country/Study/Group	Age		Pretest				Posttest				
			IQ		ABC		IQ		ABC		
	Mean	SD	Mean	SD	Mean	SD	Mean	SD	Mean	SD	
Howard et al. (2005)											
IBI (clinic)	30.9	5.2	58.5	18.2	70.5	11.9	89.9	20.9	81.3	11.1	
Comparison (AP) ^d	37.4	5.7	53.7	13.5	69.8	10.5	62.1	19.6	69.1	12.9	
Control (GP) ^e	34.6	6.5	59.9	14.8	71.6	10.5	68.8	15.3	68.3	9.9	
Cohen et al. (2006)											
IBI (clinic)	34.4	5.4	62.0	16.4	64.0	8.4	81.1	21.8	79.5	13.4	
Control	33.2	3.7	59.4	14.7	71.9	11.5	65.9	16.5	70.7	13.3	

(Table 1 continued)

Table 1. Extended Continued

Intensity		<i>n</i>	Gender		Design/Assignment/ Inclusion ^a	Comments ^b
Hr	Mon.		M	F		
25–40	14	29	25	4	QCT/parental preference and IEP teams	IBI: Multiple settings (home, school, and community) 25–30 hr per week under 3 years of age 35–40 hr per week over 3 years of age Autism educational programming: public classroom for children with autism 1:1 or 1:2 staff:child ratio 25–30 hr per week of intervention, supervision by special education teacher Intervention eclectic (PECS, SIT, TEACCH, DTT) 7 children received 1–2 session per week of speech therapy Generic educational programming: local community special education classrooms Average of 15 hr per week intervention, 1:6 staff:child ratio 13 children received speech and language therapy 1–2 times per week INDA Community-nonuniversity setting Community services selected by family In Control Group 1, child had an Early Start Autism Intervention Program 9 hr a week 2 children home-based development program 1–4 hr a week 17 special day class eclectic, ratio 1:1 to 3:1, 3–5 days a week for up to 5 hr Speech, behavioral, and occupational therapies 0–5 hr per week 3 where mainstreamed for up to 45 minutes a day INDA
25	13	16	13	3	Included if CA < 48 months	
15	15	16	16	0		
35–40		24	18	3	QCT/parental preference	Community-nonuniversity setting Community services selected by family In Control Group 1, child had an Early Start Autism Intervention Program 9 hr a week 2 children home-based development program 1–4 hr a week 17 special day class eclectic, ratio 1:1 to 3:1, 3–5 days a week for up to 5 hr Speech, behavioral, and occupational therapies 0–5 hr per week 3 where mainstreamed for up to 45 minutes a day INDA
		24	17	4	Included if CA < 48 months and ratio IQ > 35	

Table 1. Continued

Country/Study/Group	Age		Pretest				Posttest			
			IQ		ABC		IQ		ABC	
	Mean	SD	Mean	SD	Mean	SD	Mean	SD	Mean	SD
Australia										
Birnbrauer & Leach (1993)										
IBI (clinic)	38.1	7.1	45.3	17.9	47.5		57.6	18.7	41.0	5.1
Control	33.2	10.3	45.0	9.4	51.5		43.2	15.0	42.5	4.9
Israel										
Ben-Itzhak & Zachor (2007)										
IBI (clinic)	25.9	3.2	71.4	18.8	65.9	7.1	82.9	23.2	90.7	12.3
Norway										
Eikeseth et al. (2002)										
IBI (clinic)	66.3	11.3	61.9	11.3	55.8	9.0	79.1	18.1	67.0	16.3
Comparison	64.8	9.9	65.2	15.0	60.0	13.2	68.9	18.8	60.2	11.7
Eldevik et al. (2006)										
IBI (clinic)	53.1	9.5	41.0	15.2	52.5	3.9	49.2	16.6	52.4	9.2
Comparison	45.1	16.5	42.8	13.0	50.1	9.2	38.5	15.5	44.6	7.5
United Kingdom										
Hayward et al. (2009)										
IBI (clinic)	35.7	6.2	53.5	15.1	62.3	6.8	72.0	19.6	68.3	14.5
IBI (parent)	34.4	5.7	54.7	15.3	65.1	10.4	69.7	22.9	72.5	17.3
Remington et al. (2007)										
IBI (clinic)	35.7	4.0	61.4	16.7	60.2	5.8	73.5	27.3	61.5	15.4
Control	38.4	4.4	62.3	16.6	57.0	6.8	60.1	27.8	54.6	13.1

^aUCT = uncontrolled clinical trial, QCT = quasiexperimental controlled clinical trial, RCT = randomized controlled clinical trial. INDA = individual data obtained from author. VABS = Vineland Adaptive Behavior Scales, ABC = adaptive behavior composite. ^cIntensive behavioral intervention.

intake, and intensity of treatment (median split of intensity at 36 hr per week). To protect against some errors of statistical inference, we centered all variables following the guidelines suggested by Kraemer and Blasey (2004). Thus, the binary independent variable (high or low intensity of treatment) was recoded as either $+1/2$ or $-1/2$ and

all other independent variables (age, IQ, and ABC scores at intake), by subtracting the median value. In addition to the main predictor variables, we added an interaction analysis between the main predictors. This was done by generating product terms from the centered variables. For IQ change, we included interaction terms for age and IQ at

Table 1. Extended Continued

Intensity		Gender		Design/Assignment/ Inclusion ^a	Comments ^b	
Hr	Mon.	<i>n</i>	M			F
19	22	9	5	4	QCT/parent willingness and geographical Included if CA between 24 and 48 months	Untestable subjects set to IQ of 30 Ratio scores computed for the rest of subjects Scores posttreatment are deviation IQ used where available Ratio VABS scores calculated both pre and post INDA
–	24	5	5	0		
35	12	21	20	1	UCT/enrollment center	Only children available for Year 2 follow-ups included here 5 children 23 months and 2 children 21 months at intake INDA
28	12	13	8	5	QCT/staff availability Included if CA between 48 and 84 months and IQ ≥ 50	Comparison received eclectic treatment of similar intensity INDA
29	14	12	11	1		
13	20	13	10	3	QCT/archival date Included if CA < 72 months	Comparison received eclectic treatment of similar intensity 4 subjects from comparison group taken out here because included in Eikeseth et al. (2002) INDA
12	23	11	10	1		
37	13	23	19	4	QCT/geographical Included if CA < 42 months	INDA
34	14	21	15	6		
26	24	23	18	5	QCT/parent preference Included if CA between 30 and 42 months	Control group received TAU, special school, mainstream or mix, but little or no 1:1, speech therapy, TEACCH etc. INDA
16	24	21	18	3		

intake, age at intake and intensity of intervention, and IQ at intake and intensity. For change in ABC, we included interaction terms for age and ABC scores at intake, age at intake and intensity of intervention, and ABC at intake and intensity of intervention.

Results

The proportion of children in intensive behavioral intervention, control, and comparison groups achieving reliable change in IQ and ABC is displayed in Figure 2. Each bar on the graph in

Table 2. Available Number of Subjects and Demographics of Entire Sample and Subgroups

Group	Age				Intelligence				Adaptive behavior			
	<i>n</i>	Mean	<i>SD</i>	Range	<i>n</i>	Mean	<i>SD</i>	Range	<i>n</i>	Mean	<i>SD</i>	Range
Behavioral treatment (<i>n</i> =309)	278	38.0	11.4	16–84	286	55.6	18.2	17–120	252	60.3	10.9	26–95
Control (<i>n</i> =309)	95	36.5	7.1	18–72	105	54.8	17.1	19–97	73	65.0	11/6	45–113
Comparison (<i>n</i> =39)	39	47.6	15.9	21–84	39	47.6	15.9	21–84	39	61.2	13.7	37–96
Total (<i>N</i> =453)	412	38.5	11.5	16–84	430	55.3	17.7	17–120	362	61.3	11.5	26–113

Figure 2 represents an individual child's change in test score. These have been sorted from the highest negative to the highest positive change. A reference line on the y-axis shows the criterion for reliable change. Overall, 83 of the 279 children in the intensive behavioral intervention group (29.8%) achieved reliable change in IQ and 51 of 248 achieved reliable change in ABC scores (20.6%). In the control group, 9 of 104 achieved reliable change in IQ (8.7%), and 4 of 70 achieved reliable change in ABC scores (5.1%). In the comparison interventions group, 1 of 39 children achieved reliable change in IQ (2.6%), and 2 of 39 achieved reliable change in ABC scores (5.7%). We compared the proportions in the three groups statistically using 3×2 chi-square tests. There was a significant difference in the proportions achieving reliable change for IQ, $\chi^2(2, N = 422) = 29.11, p < .001$, and for ABC, $\chi^2(2, N = 357) = 11.81, p = .003$. Examination of the standardized residuals in the six cells of these two analyses revealed that there were more children than expected achieving reliable change in the intensive behavioral intervention group, and fewer children than expected achieving this change in the two other groups. Exploratory 2×2 chi-square comparisons between the control and comparison group for IQ and ABC change revealed no difference between these two groups, $\chi^2(1, N = 130) = 2.06, p < .151$ and $\chi^2(1, N = 96) = .141, p = .707$, respectively.

Because the chi-square comparisons showed that there were no significant differences in outcome between the control and comparison groups, we combined them to carry out the individual participant data meta-analysis focusing on the number needed to treat and absolute risk reduction for intensive behavioral intervention. The number needed to treat was computed to be 5, 95% CI = 3.4, 6.3, for achieving a reliable change in IQ and 7, 95% CI = 4.5, 9.8, for

achieving reliable change in ABC scores, which translates to an absolute risk reduction of 23%, 95% CI = 16.0%, 29.6%, and 16%, 95% CI = 10.2%, 22.3%, respectively, in favor of the intensive behavioral intervention group. The number needed to treat and absolute risk reduction for IQ and ABC, along with the 95% confidence intervals for the individual studies (i.e., the controlled studies in which there is a comparison or control group against which to calculate an effect size) are shown in Tables 3 and 4. At the level of individual studies, there is considerable variability in effect sizes, and many of the individual studies were focused on small samples and, therefore, were underpowered.

The multiple regression analyses for prediction of IQ and ABC change are summarized in Table 5. A graphical analysis of residuals showed the assumptions of normality and equal variance approximately held. Overall, the models explained a statistically significant, though small, proportion of the variance for both IQ change, $F(4, 211) = 5.22, p < .001, R^2 = .090$, adjusted $R^2 = .073$, and ABC change, $F(4, 213) = 14.45, p < .001, R^2 = .213$, adjusted $R^2 = .199$. The results from the regression analyses showed that high intervention intensity was the only variable that independently and positively predicted both IQ and ABC gain. In addition, ABC at intake and IQ at intake predicted gains in ABC. Those children with lower ABC scores at intake had larger ABC change over 2 years, whereas higher IQ at intake predicted larger ABC gains. No interaction terms were statistically significant independent predictors of IQ or ABC change.

Discussion

Despite the recognized difficulties of obtaining individual participant data over a long time period (20+ years of research) (Cooper & Patall,

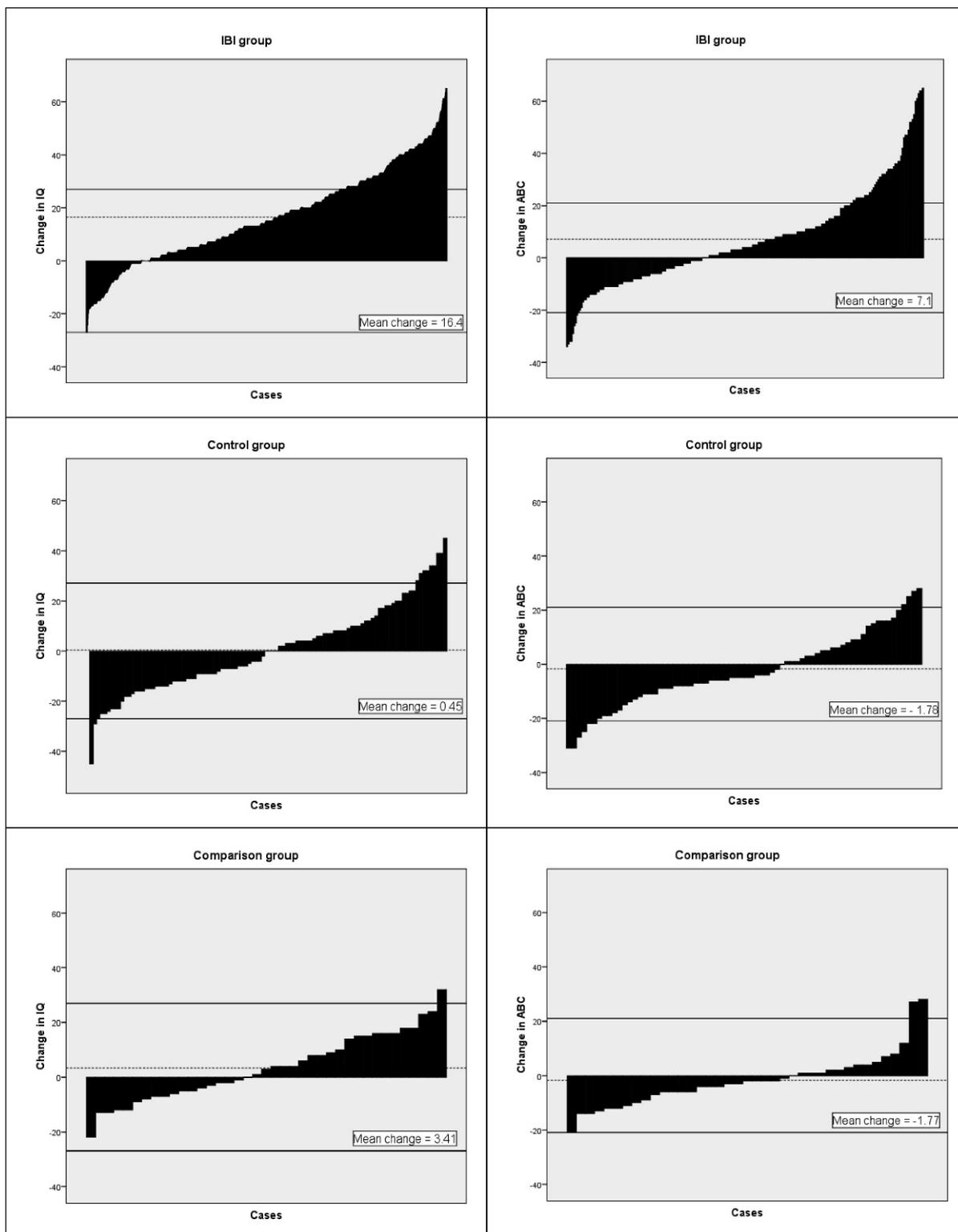


Figure 2. Bars indicate changes in IQ and ABC scores for children in the IBI, control, and comparison groups. The lines at ± 27 IQ points and ± 21 Adaptive Behavior Composite (ABC) points show the criteria for reliable change. The dotted line shows the mean change for the group.

Table 3. Number of Children Meeting Reliable Change Criteria: Outcome Intelligence

Study/Group	Outcome intelligence ^a					
	RCI+	RCI–	NNT	95% CI NNT/NNH	ARR (%)	95% CI (%)
Lovaas (1987)						
IBI ^b	9	10	3	1,5–5,7	42.0	17.5–66.7
Control	1	18				
Eikeseth et al. (2008)						
IBI	0	10	5	2,2–575.3	23.1	0.2–46.0
Comparison	3	12				
Birnbrauer & Leach (1983)						
IBI	2	7	5	NNT 2,0 to NNH 20,2 ^d	22.2	–4.9–49.4
Control	0	5				
Smith et al. (2000) (RCT) ^c						
IBI	6	9	3	1,5–6,6	40.0	15.2–64.9
Control	0	13				
Eldevik et al. (2006)						
IBI	0	13		—	0.0	0.0
Comparison	0	11				
Smith et al. (1997)						
IBI	1	10	11	NNT 3,8 to NNH 12.7 ^d	9.1	–7.9–26.1
Control	0	10				
Howard et al. (2005)						
IBI	14	11	3	1,5–10	37.3	10.0–64.5
Control	3	13				
Howard et al. (2005)						
IBI	14	11	3	1,4–3,7	49.8	27.0–72.5
Comparison	1	15				
Cohen et al. (2006)						
IBI	9	12	4	1,7–12,6	32.9	7.9–57.8
Control	2	18				
Remington et al. (2007)						
IBI	5	18	14	NNT 3,3 – NNH 6,6 ^d	7.5	–15.1–30.0
Control	3	18				

^aRCI = reliable change index, the plus sign signifies that criterion for reliable change was met; the minus sign means that criterion was not met. NNT = number needed to treat, NNH = number needed to harm, ARR = absolute risk reduction, CI = confidence interval. ^bIntensive behavioral interventions. ^cRandomized controlled clinical trial. ^dBecause the 95% CI for the absolute risk reduction extends from a negative number where treatment may harm (NNH) to a positive number where treatment may benefit, it is hard to compute a 95% CI for the NNT. This means that we cannot say with 95% certainty whether the intervention is harmful, has no effect, or is helpful compared to control. What we can say in this instance is that we can be 95% certain that one of these statements is true: The experimental treatment is harmful (compared to control), and the NNH is greater than x. The experimental treatment is helpful (compared to control), and the NNT is greater than y. Expressed as NNT y to ∞ (indefinitely) to NNH x (adapted from Altman, 1998).

Table 4. Number of Children Meeting Reliable Change Criteria: Outcome Adaptive Behavior

Study/Group	Outcome adaptive behavior ^a					
	RCI+	RCI–	NNT	95% CI NNT/NNH	ARR (%)	95% CI (%)
Eikeseth et al. (2002)						
IBI ^b	4	9	4	1.8–17.6	30.8	5.7–55.9
Comparison	0	12				
Bimbrauer & Leach (1993)						
IBI	0	9	–5	NNT 6.6 ~ NNH 1,8 ^c	–20/0	–15.1–55.1
Control	1	4				
Smith et al. (2000) RCT ^c						
IBI	4	11	4	2,0–23.3	26.6	15.2–64.8
Control	0	13				
Eldevik et al. (2006)						
IBI	1	12	13	NNT 4,5 ~ NNH 14,7 ^c	7.7	–6.8–22.2
Comparison	0	11				
Howard et al. (2005)						
IBI	5	18	5	2,6–20,5	21.7	4.9–38.6
Control	0	13				
Howard et al. (2005)						
IBI	5	18	11	NNT 3.1 ~ NNH 7,1 ^c	9.2	–14.1–32.6
Comparison	2	14				
Cohen et al. (2006)						
IBI	4	15	11	NNT 3,0 ~ NNH 7,4 ^c	9.9	–13/4–33.3
Control	2	16				
Remington et al. (2007)						
IBI	2	21	26	NNT 5,4 ~ NNH 9,3 ^c	3.9	–10.8–18.6
Control	1	20				

^aRCI = reliable change index, the plus sign signifies that criterion for reliable change was met; the minus sign means that criterion was not met. NNT = number needed to treat, NNH = number needed to harm, ARR = absolute risk reduction, CI = confidence interval. ^bIntensive behavioral interventions. ^cRandomized controlled clinical trial. ^dBecause the 95% CI for the absolute risk reduction extends from a negative number where treatment may harm (NNH) to a positive number where treatment may benefit, it is hard to compute a 95% CI for the NNT. This means that we cannot say with 95% certainty whether the intervention is harmful, has no effect, or is helpful compared to control. What we can say in this instance is that we can be 95% certain that one of these statements is true: The experimental treatment is harmful (compared to control), and the NNH is greater than x. The experimental treatment is helpful (compared to control), and the NNT is greater than y. Expressed as NNT y to ∞ (indefinitely) to NNH x (adapted from Altman, 1998).

2009), we were able to gather such data for each of the 16 evaluation studies of intensive behavioral intervention identified via a systematic review. Only data from one of Lovaas' (1987) original control groups were unavailable. When we compared the intensive behavioral intervention group with control and comparison groups, an individual participant data meta-analysis showed meaningful differences in outcomes for children

with autism in favor of intensive behavioral intervention. For IQ, the number needed to treat was 5 (absolute risk reduction = 23%), and for the ABC, the number needed to treat was 7 (absolute risk reduction = 16%). Given that the data for this individual participant data meta-analysis were identified via a systematic review, they might be considered a benchmark against which to evaluate future intensive behavioral intervention outcome

Table 5. Regression Analysis of Predictors of IQ and Adaptive Behavior Composite (ABC) Gain in the Intensive Behavioral Intervention

Predictor	IQ gain	
	β	p
IQ		
Main effects		
IQ at intake	-.135	.031
ABC ^a at intake	.128	.054
Age at intake	-.069	.282
Intensity	.266	.000
Interactions		
Age at intake \times IQ at Intake	-.021	.718
Age at Intake \times Intensity	-.049	.382
IQ at Intake \times Intensity	.014	.811
ABC		
Main effects		
IQ at intake	.363	.031
ABC at intake	-.342	.054
Age at intake	-.038	.282
Intensity	.217	.000
Interactions		
Age at intake \times ABC at Intake	.102	.132
Age at Intake \times Intensity	.058	.365
ABC at Intake \times Intensity	.190	.005

^aAdaptive Behavior Composite.

studies as well as to audit the outcomes achieved in clinical practice. Such data have not been previously available in the field.

The effect sizes obtained from the individual participant data meta-analysis compare favorably to psychological and medical treatments for common disorders such as major depression (number needed to treat between 3 and 5), obsessive compulsive disorders (number needed to treat between 4 and 5), and bulimia nervosa (number needed to treat = 9) (Pinson & Gray, 2003). We have not been able to locate published number needed to treat or absolute risk reduction data for other interventions for autism. The decision to offer interventions cannot be made by looking at the number needed to treat score in isolation; one would also need to know the intervention costs, long-term economic and social savings, and resources required. Also, any side effects of intervention would be important to document. Full data on these variables are not currently available in the field of autism. Howev-

er, it is informative to note that there appears to be no additional negative psychological impact on family members associated with intensive behavioral intervention (Hastings, 2003; Hastings & Johnson, 2001; Remington et al., 2007). Furthermore, autism-specific eclectic preschool services may cost no less than home-based intensive behavioral intervention (Magiati et al., 2007).

The present analysis provides evidence that intensive behavioral intervention is an evidence-based intervention for children with autism. According to the criteria developed by the Oxford Centre for Evidence Based Medicine (2009), the evidence for intensive behavioral intervention for young children with autism is at Level 1b. This level requires evidence from at least one well-designed randomized controlled study and evidence from systematic reviews. Level 1a (the highest level of evidence) would require a systematic review of several randomized controlled trials showing homogeneity in results. Similarly, the intensive behavioral intervention evidence base meets the criteria for the evidence-based practices in special education proposed by Gersten et al. (2005). These criteria require at least four acceptable quality studies or two high quality studies supporting the practice and a weighted effect size significantly greater than zero (e.g., Eikeseth, 2009), one high quality study (Smith et al., 2000) and four acceptable quality studies (Cohen et al., 2006; Eikeseth et al., 2002; Howard et al., 2005; Remington et al., 2007). Eldevik et al. (2009) found that all of these studies had a weighted effect size significantly greater than zero.

Combined with the earlier meta-analysis of controlled studies reported by Eldevik et al. (2009) based on effect sizes calculated using individual participant data, the present individual participant data meta-analysis completes the two meta-analysis steps advocated by Cooper and Patall (2009). The evidence from the present study also extends the number of studies included in the Reichow and Wolery (2009) aggregated data mean change effect size meta-analysis and, like the Eldevik et al. study, adds a quantitative dimension to earlier systematic reviews (Howlin et al., 2009; Reichow & Wolery, 2009).

An individual participant data analysis vastly increases the power to detect intervention effects (Cooper & Patall, 2009), establishing estimates with reduced error. However, it is clear from Tables 3 and 4 that there is considerable variability in the estimates of effect sizes (number needed

to treat and absolute risk reduction) at the level of individual studies. These tables only include controlled studies that could be used to generate study level effect sizes (i.e., pretest–posttest single group designs are excluded from these tables). In addition, several studies include only very small samples within which one or two children reaching, or not quite reaching, criteria for reliable change on either IQ or ABC can have a large impact on the computed effect sizes. In several individual studies (especially for ABC outcomes), the confidence intervals obtained for the effect sizes precluded any conclusion of likely positive gain or harm for the children in that study. These data have been provided for information purposes and to allow researchers to draw their own conclusions about the variability in outcomes within individual studies. However, these data also confirm the importance of carrying out individual participant data meta-analysis across studies in drawing conclusions about the evidence base of an intervention.

In addition to the variability summarized in Tables 3 and 4, in applying the general common elements of intensive behavioral intervention defined by Green et al. (2002), we may risk combining quite different interventions. For example, we made no distinction between center-based, community-based, or home-based programs. We know that the level and frequency of supervision will have varied between studies, although we did not have access to relevant data. Furthermore, separate intensive behavioral intervention programs are likely to stress the use of techniques differently; some may be based heavily on discrete trial training; others, on incidental teaching; others, on pivotal response training; and still others, on verbal behavior and natural environment teaching. As the field develops, it will be important to complete further meta-analyses based on evaluation studies of interventions sharing a more restricted set of features. At the present time, too few studies are available to enable this task.

We also conducted a large sample analysis of the correlates of outcome within the intensive behavioral intervention group of 309 children. The results from these regression analyses show that high intervention intensity was the only variable that independently predicted both IQ and ABC gain. In both cases, high intensity (36+ weekly intervention hours) was associated with larger gains. In addition, ABC at intake and IQ at

intake predicted gains in ABC. Those children with lower ABC scores at intake had larger ABC change over 2 years (perhaps indicating ceiling effects for those who start with higher ABC scores at intake), whereas higher IQ at intake predicted larger ABC gains. No interaction terms were statistically significant independent predictors of IQ or ABC change. These findings generally confirm those of previous research that suggest intensity and intake ability may be associated with outcome in intensive behavioral intervention (Eikeseth et al., 2007; Harris & Handleman, 2000; Lovaas, 1987; Remington et al., 2007). Interestingly, despite the considerable sample size, no hypothesized interactions between variables predicted outcome. It is still likely to be important to explore interactions between predictors of outcome in future research where sample size permits because such interactions may tell us a great deal about the ideal conditions for positive outcomes for intensive behavioral intervention. Our conclusions are limited by the lack of available data on correlates of outcome and also the likely lack of validity of the coding of intervention intensity. There is no substitute for the systematic exploration of moderator effects built into the design of intervention studies (Kraemer, Frank, & Kupfer, 2006), and this is a priority for future intensive behavioral intervention research.

One potential difficulty with our research is that the criteria used to calculate whether an individual child's changes in test scores were reliable might be considered conservative. The reliable change criteria that were computed in the present study required a substantial change in IQ (27 points) and ABC (21 points), arguably representing a significant practical gain, reflecting improvements in the potential for independent living, improved quality of life, a reduced need for professional support, and a reduced economic cost for long-term care and habilitation (J. Jacobson, Mulick, & Green, 1998; Jarbrink & Knapp, 2001). Under many circumstances, a change equivalent to one *SD* would be considered substantial, especially when using standardized and norm-referenced instruments, such as intelligence scales and the VABS (Weinberg, 1989). Our approach emphasizes the importance of data specific to young children with autism in considering change as a result of intervention. In fact, making the assumption that data from normative samples will apply for children with

autism may lead to overestimates of the impact of an intervention.

Perhaps the most significant limitation of the present individual participant data meta-analysis is the quality of the studies entering the review. We applied several important quality control criteria (e.g., definition of intensive behavioral intervention used, quality of outcome measurement), but we did not exclude studies on the basis of research design (apart from case studies). Specifically, there is a lack of true random assignment to groups (except for two studies), the use of different assessment instruments both within and across studies, and the lack of measures of intervention fidelity. Furthermore, there is considerable variability in the duration of treatment (although we standardized that to a greater degree than would have been possible relying only on published aggregated data from each study). Thus, our results should be viewed as preliminary, and future researchers conducting meta-analyses will need to incorporate research quality selection criteria when the body of randomized studies available for analysis is larger.

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Correspondence regarding this article should be sent to Sigmund Eldevik, Akershus University College. BP. 423, Lillestrom, 2002, Norway. E-mail: eldevik@online.no

The Cost-Effectiveness of Expanding Intensive Behavioural Intervention to All Autistic Children in Ontario

Rentabilité de l'étendue des services d'intervention comportementale intensive à tous les enfants autistes de l'Ontario

In the past year, several court cases have been brought against provincial governments to increase funding for Intensive Behavioural Intervention (IBI). This economic evaluation examines the costs and consequences of expanding an IBI program.



by SANOBER S. MOTIWALA, MSC
*Department of Health Policy, Management and Evaluation
University of Toronto, ON*

SHAMALI GUPTA, MSC
*Department of Health Policy, Management and Evaluation
University of Toronto, ON*

MEREDITH B. LILLY, HON BA
*Department of Health Policy, Management and Evaluation
Fellow, CIHR Program in Healthcare, Technology and Place
University of Toronto, ON*

Sanobar S. Motiwala, Shamali Gupta, Meredith B. Lilly, Wendy J. Ungar and Peter C. Coyte

WENDY J. UNGAR, PHD

Scientist, Population Health Sciences, Hospital for Sick Children

Adjunct Scientist, Institute for Clinical Evaluative Sciences

Assistant Professor, Department of Health Policy, Management and Evaluation

University of Toronto, ON

PETER C. COYTE, PHD

Health Services Chair

Canadian Health Services Research Foundation and Canadian Institutes of Health Research

Professor, Health Economics, Department of Health Policy, Management and Evaluation

University of Toronto, ON

Abstract

Intensive Behavioural Intervention (IBI) describes behavioural therapies provided to autistic children to overcome intellectual and functional disabilities. The high cost of IBI has caused concern regarding access, and recently, several court cases have been brought against provincial governments to increase funding for this intervention. This economic evaluation assessed the costs and consequences of expanding an IBI program from current coverage for one-third of children to all autistic children aged two to five in Ontario, Canada. Data on the hours and costs of IBI, and costs of educational and respite services, were obtained from the government. Data on program efficacy were obtained from the literature. These data were modelled to determine the incremental cost savings and gains in dependency-free life years. Total savings from expansion of the current program were \$45,133,011 in 2003 Canadian dollars. Under our model parameters, expansion of IBI to all eligible children represents a cost-saving policy whereby total costs of care for autistic individuals are lower and gains in dependency-free life years are higher. Sensitivity analyses carried out to address uncertainty and lack of good evidence for IBI efficacy and appropriate discount rates yielded mixed results: expansion was not cost saving with discount rates of 5% or higher and with lower IBI efficacy beyond a certain threshold. Further research on the efficacy of IBI is recommended.

Résumé

L'intervention comportementale intensive (ICI) décrit les thérapies comportementales fournies aux enfants autistes pour les aider à surmonter leurs déficiences intellectuelles et fonctionnelles. Les coûts élevés de cette intervention ont soulevé des préoccupations quant à l'accès et, récemment, plusieurs poursuites judiciaires ont été intentées contre les gouvernements provinciaux en vue d'amener ces derniers à augmenter le finance-

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ment accordé à l'ICI. Cette analyse économique visait à évaluer les coûts et les conséquences de l'élargissement de la portée d'un programme d'ICI pour le rendre accessible à tous les enfants autistes âgés de deux à cinq ans en Ontario, au Canada – au lieu du tiers des enfants comme c'est le cas actuellement. Les données sur les heures et les coûts liés à l'ICI, ainsi que sur les coûts des services éducatifs et de relève, ont été obtenues auprès du gouvernement. Les données sur l'efficacité du programme ont été tirées de la littérature. Ces données ont été modélées afin de déterminer les économies supplémentaires et les années de vie autonome gagnées. L'élargissement de la portée du programme actuel a permis de réaliser des économies de 45 133 011 \$ CAN en 2003. Selon les paramètres de notre modèle, étendre l'ICI à tous les enfants admissibles constitue une mesure de réduction des dépenses en vertu de laquelle les coûts totaux des soins fournis aux enfants autistes sont moins élevés et les gains d'autonomie sont plus élevés. Les analyses de sensibilité effectuées pour aborder l'incertitude et le manque de données solides corroborant l'efficacité de l'ICI et les rabais appropriés pour cette dernière ont donné des résultats mixtes : l'élargissement de la portée de l'ICI ne permet pas de réaliser des économies avec des rabais de 5 p. cent ou plus ou avec un seuil d'efficacité en deçà d'un certain niveau. Nous recommandons d'effectuer des travaux de recherche plus poussés sur l'ICI.

AUTISM IS AN EARLY-ONSET DEVELOPMENTAL DISABILITY CHARACTERIZED by impairments in social interaction, abnormal verbal and non-verbal communication, repetitive, stereotyped behaviour and resistance to change (Howlin 1998; American Psychiatric Association 1994). Most cases are diagnosed by three years of age, with a male–female ratio of 3:1 (Ontario Ministry of Community, Family and Children's Services [MCFCS] 2000). The reported prevalence of autism in Ontario almost doubled between 1996 and 1998, with the 1998 prevalence being 2.09 per 1,000 children aged five and younger (Ontario Health Insurance Program [OHIP] 2000). The etiological cause of autism is believed to be dysfunction of the right hemisphere of the brain, which is responsible for appropriate visual–spatial and emotional interactions (Gillberg and Coleman 2000).

Intensive Behavioural Intervention (IBI) is the general term for behavioural therapies provided to autistic children to overcome their intellectual and functional disabilities. Several variants of IBI and non-IBI therapies have been reported, but strong evidence is lacking regarding the effectiveness of many of these approaches. No single form of behavioural intervention is appropriate for all individuals with autism (Dawson and Osterling 1997). IBI typically involves one-on-one training provided by a therapist, in which children are trained to respond to environmental changes, understand and use language and interact appropriately with others in social settings

(Dawson and Osterling 1997). Positive reinforcement is used to internalize appropriate behaviours. Success of IBI is believed to correspond to the intensity and duration of the treatment – between 20 and 40 hours per week of one-on-one therapy, for a minimum of two years, is generally believed to yield optimal results (MCFCS 2000; Lovaas 1987). Beyond a minimum threshold of 20 hours per week, there is little agreement in the peer-reviewed literature as to the exact number of hours required to achieve the most favourable results (MCFCS 2000; Dawson and Osterling 1997; Bassett et al. 2000; Sheinkopf and Sigel 1998; Smith 1999; Ludwig and Harstall 2001). Annual IBI costs range from \$40,000 to \$75,000 per child in 2003 Canadian dollars, depending on the number of treatment hours provided and other factors, including administrative costs and training (Ontario Ministry of Children's Services [MCS] and Ontario Ministry of Community and Social Services [MCSS] 2003; Jacobson and Mulick 2000; Jacobson et al. 1998; Hildebrand 1999; Freeman 1997).

IBI outcomes are generally categorized by level of functioning, assessed at the end of the intervention period. "Normal-functioning" individuals integrate into the community, receive schooling in mainstream classrooms and live independently as adults. "Semi-dependent" and "very dependent" individuals make partial and minimal gains, respectively, and continue to rely on social assistance throughout their lifetime (Lovaas 1987; Freeman 1997; Rutter 1996; Howlin 1997; Howlin et al. 2004). The most optimistic estimates available in the literature suggest that without receiving any form of intervention, as many as 25% of autistic individuals live normal lives, 25% are moderately disabled and 50% are severely compromised (Freeman 1997). However, other studies have reported lower rates of normalization without intervention (Rutter 1996; Howlin 1997). Success rates of IBI and similar interventions vary.

A highly publicized and controversial study, conducted by Lovaas (1987), reported a large proportion of children (up to 47%) achieving normal intellectual and educational functioning at the end of the intervention. However, Lovaas's primary study and its follow-up (McEachin et al. 1993) have been criticized for their methodological limitations, particularly, exclusion of the poorest-functioning 15% of referred subjects, the non-random assignment of children to treatment groups and the statistically significant difference in sex ratios between the treatment and control groups. These limitations have led to concerns regarding the validity of Lovaas's findings (Bassett et al. 2000).

In Canada, funding for IBI varies across provinces, but most provincial governments offer some support for IBI to children diagnosed with autism up to a certain age. As a result of high costs of treatment, several lawsuits have been launched by families of autistic children, rallying for increased government funding for IBI. In most cases, rulings have been favourable for the families, requiring governments to increase funding for IBI. In contrast, the Supreme Court of Canada recently ruled favourably in an appeal from the British Columbia government, denying increased funding for

IBI on the grounds that the therapy did not constitute “medically necessary” care as defined by the *Canada Health Act*.

In Ontario, the government currently funds up to three years of IBI for approximately a third of autistic children younger than six years of age (OHIP 2000; MCSS 2002). The Ontario government does not promote any particular form of IBI. It has contracted with a private organization (Behaviour Institute, Hamilton) that delivers training to regional service providers, who in turn are contracted through a competitive tendering process. In its provincial program guidelines for IBI, the government lists principles and teaching methods that regional providers are expected to follow, which include, where appropriate, one-on-one training, task analysis, positive reinforcement and small-group instruction (MCFCS 2000). Eligibility for IBI, duration and intensity of treatment are determined through formal assessment, with allocation of services geared towards children with more severe forms of autism (MCFCS 2000). Earlier this year, the Superior Court of Ontario ruled in favour of the plaintiffs in a class-action lawsuit against the Ontario government, challenging the termination of public funding for IBI at the age of six. The decision is currently being appealed.

The purpose of this study was to conduct a cost-effectiveness analysis to evaluate the expansion of the IBI program to all autistic children in Ontario from two to five years of age, commencing in 2003. We included costs incurred only by the government and excluded all other costs, for example, those incurred by autistic individuals, their families and employers. The government’s perspective was employed for the analysis because it is highly relevant to ongoing legal and policy debates across the country. The provision of IBI in this model was limited to children aged two to five because (1) IBI is believed by many to be most effective when provided at an early age (MCFCS 2000); (2) currently, the Ontario government funds IBI only for children under the age of six (MCS and MCSS 2003); and (3) previous economic analyses carried out in other jurisdictions have limited IBI provision to children of similar ages (Jacobson et al. 1998; Hildebrand 1999). Thus, the present model would facilitate comparisons.

Methods

Including costs incurred only by the government, we developed a model that reflects the current public provision of autism services in Ontario. The prevalence of autism in Ontario, or the cohort size for this study ($n = 1,309$), was calculated as the sum of the number of children receiving IBI ($n = 485$), the number of children eligible but wait-listed for IBI ($n = 91$) and the number of children waiting for an assessment, multiplied by the proportion of assessed children who have historically been deemed eligible for IBI ($n = 952 \times 0.77$). The three comparison groups were (1) *Status Quo* provision, (2) *Expansion* of IBI services and (3) *No Intervention*. *Status Quo* was based on the current provision of autism services by the provincial government, whereby 37% of

children with autism aged two to five ($n = 485$) receive up to three years of IBI for 23 hours per week on average, while the remainder ($n = 824$) do not receive IBI. While the majority of children currently eligible for IBI in Ontario receive it for less than three years because of diagnostic delays and waiting lists, our study was based on the assumption that all children eligible for these services would receive them for a fixed three-year duration. Under *Expansion*, IBI was provided to all autistic children ($n = 1,309$) for three years at 23 hours per week. Under the third scenario, *No Intervention*, IBI was not provided to any of the 1,309 children in the cohort. Although this scenario represents an unlikely regression from the current situation in Ontario, it makes our findings relevant for jurisdictions where IBI may not be currently publicly funded.

Efficacy rates

Under all three scenarios, children were categorized according to their levels of functioning – normal, semi-dependent and very dependent – upon completion of IBI until the age of 65 (Table 1) (Jacobson et al. 1998; Hildebrand 1999). Efficacy rates for *No Intervention*, the cohort that received no IBI, were based on published literature (Freeman 1997; Howlin et al. 2004; Green et al. 2002). It was assumed that 25% attain normal functioning, 25% are semi-dependent and 50% are very dependent without receiving IBI (Freeman 1997). The figures from Freeman (1997) are the most optimistic reported in the literature; they match closely more recent estimates of adult functioning by Howlin et al. (2004), which are slightly lower. Although many studies report even lower rates of normalization (Rutter 1996; Howlin 1997), we selected the highest published rates to investigate the cost-effectiveness of IBI from a best-case scenario, thereby increasing the robustness of our model.

Because of ongoing controversy regarding the reported efficacy of Lovaas's treatment and other forms of behavioural intervention (Dawson and Osterling 1997; Bassett et al. 2000; Sheinkopf and Sigel 1998; Smith 1999; Ludwig and Harstall 2001; Green et al. 2002), we assigned IBI efficacy rates that were more conservative than those reported for Lovaas's intervention (1987) and its replications (McEachin 1993; Sallows and Graupner 2001). The efficacy rates for *Expansion* were assumed to be 30% normal, 50% semi-dependent and 20% very dependent. *Status Quo* efficacy was based on a weighted average of 824 children receiving no IBI (efficacy equivalent to *No Intervention*) and 485 children receiving IBI (efficacy equivalent to *Expansion*) for three years. The resultant efficacy rates for *Status Quo* were 26.9% normal, 34.3% semi-dependent and 38.9% very dependent.

Cost Data Sources

All costs in the model were converted to 2003 Canadian dollars using growth in the

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consumer price index from the period when the underlying data were available, and were estimated for individuals from age two to 65.

The Ontario Ministry of Children's Services and Ontario Ministry of Community and Social Services (2003) reported the annual cost of IBI as \$75,670 per child aged two to five, based on 23 hours per week of therapy. This figure represents the aggregate cost of the IBI program incurred by the Ontario government and includes the training costs of IBI therapists, contractual payments to service providers, and salaries, benefits and overhead costs incurred by provincial civil servants. Average wage rates from Statistics Canada's Ontario Wage Survey (1999) were used to estimate costs for government-funded respite services and speech and language therapy (BBB Autism Support Network 2002). In all cases, costs were converted to 2003 dollars.

No autism-related costs were assumed for normal-functioning individuals after the age of five; families of semi-dependent and very dependent individuals in both the *Status Quo* and *Expansion* groups continued to receive respite services until 18 years of age. All education costs were derived from Ontario Ministry of Education documents (2000; 2001a,b,c). This ministry incurs two levels of special-education costs, Intensive Support Amount 2 (ISA 2) and Intensive Support Amount 3 (ISA 3) for semi- and very dependent individuals from five to 18 years of age.

Adult care costs for semi- and very dependent individuals were based on reports prepared by the Auditor of Ontario (MCSS 2001). Costs for adult day programs were obtained from Ontario Agencies Supporting Individuals with Special Needs (OASIS 2000). Due to limited availability of data on housing and care of autistic adults, 50% of semi-dependent individuals were assumed to live independently and 50% in public residential facilities, while all very dependent individuals were assumed to live in public residential facilities. Autistic adults are eligible for compensation through the Ontario Disability Support Program (ODSP) (Canadian Legal Information Institute 2004). ODSP benefits represent transfer payments rather than costs related directly to autism; therefore, these monthly ODSP entitlements were excluded from the model. The cost to government and other employers of administering assisted-employment programs for developmentally disabled adults was based on current programs of Human Resources Development Canada (HRDC 1999, 2001).

While healthcare utilization might be related to the level of functioning (Jarbrink and Knapp 2001), we did not have access to such data; hence, the cost-effectiveness analysis does not capture these healthcare costs. However, since utilization may increase with the level of dependence, the potential cost savings identified in this study would increase if healthcare utilization were included.

In projecting costs over the productive lifetime, a discount rate of 3.0% per annum was applied to calculate present values (Drummond et al. 1997). In sensitivity analyses, discount rates from 1.0% to 5.0% were used.

TABLE 1. Levels of functioning, efficacy rates, and dependency-free years gained for *No Intervention*, *Status Quo* and *Expansion*

LEVEL OF FUNCTIONING	DESCRIPTION	EFFICACY RATES		
		No Intervention	Status Quo*	Expansion
Normal	Mainstream classroom education; independent functioning; earn average Canadian high school graduate income as adults	25%	26.9%	30%
Semi-Dependent	Special education; respite services; 50% live independently as adults; 50% live in residential facilities; participate in day programs; earn assisted employment income as adults	25%	34.3%	50%
Very Dependent	Intensive special education; respite services; 100% live in residential facilities as adults; participate in day programs; earn assisted employment income as adults	50%	38.9%	20%
Discounted Dependency-free years gained until 65 years of age†		9.6 years	11.2 years	14.0 years

*Weighted average based on 485 children receiving IBI (efficacy: 30% normal, 50% semi-dependent, 20% very dependent) and 824 children receiving no IBI (efficacy: 25% normal, 25% semi-dependent, 50% very dependent)
 †Calculated as a weighted average based on efficacy rates for each scenario, discounted at 3% per annum

Outcomes

IBI outcomes were measured by the number of dependency-free years gained to age 65, where dependency was defined as the need for special education and other special services comprising adult day programs, disability supports and assisted employment. Normal-functioning individuals were not dependent after age five and, as a result, gained 60 dependency-free years. Very dependent individuals made minimal gains

from IBI, remained dependent throughout life and gained zero dependency-free years. Semi-dependent individuals continued to be partially dependent. Their outcome was assumed to be the midpoint between normal and very dependent functioning outcomes; they gained 30 dependency-free years. Estimated dependency-free years for the study time horizon were discounted at 3.0% per annum. The discounted number of dependency-free years gained under *No Intervention*, *Status Quo* and *Expansion* were calculated as the weighted average of dependency-free years for normal, semi- and very dependent individuals under each scenario (Table 1). The number of discounted dependency-free years per person to age 65 was 9.6 years for *No Intervention*, 11.2 years for *Status Quo* and 14.0 years for *Expansion*.

Results of the analysis were expressed in terms of incremental cost savings in present values (PVs) and gains in dependency-free years (also measured in PVs). The incremental cost analyses compared *Status Quo* to *No Intervention*, *Expansion* to *No Intervention* and *Expansion* to *Status Quo*.

Productivity costs incurred by semi- and very dependent individuals were included in a sensitivity analysis to examine costs and benefits from a partial societal perspective. Lost wages to age 65 were derived from sex-adjusted income estimates from the 1996 and 2001 Canadian censuses (Statistics Canada 1996; 2001a,b,c) and federal assisted-employment initiatives data (HRDC 1999, 2001). Potential earnings for the normal-functioning group were assumed to be equivalent to the sex-adjusted annual income of high school graduates. Semi-dependent incomes are derived from the average earnings of workers in a supported employment initiative in Newfoundland, adjusted for Ontario (HRDC 2001). Owing to lack of data, income for very dependent individuals was assumed to be 60% of the semi-dependent income. All earnings were converted to 2003 dollars. Sensitivity analyses performed also varied IBI efficacy rates and discount rates to compensate for potential estimation uncertainties and methodological controversies (Drummond et al. 1997). Additional sensitivity analyses varied the cost of IBI, adult care costs and number of dependency-years, but did not significantly affect the results presented.

Results

The annual cost during the intervention period (age two to five) for each autistic child was \$5,378 for *No Intervention*, \$33,414 for *Status Quo* and \$81,048 for *Expansion* (Table 2). The annual cost during schooling (age five to 18) was \$6,616 for normal, \$21,422 for semi-dependent and \$38,672 for very dependent individuals. No costs were incurred during adulthood for normal-functioning individuals. The annual cost during adulthood (age 18 to 65) was \$37,380 for semi-dependent adults and \$75,648 for very dependent adults. The average total discounted cost per individual, based on a weighted average of normal, semi-dependent and very dependent costs

over the study time horizon, was \$1,014,315 for *No Intervention*, \$995,074 for *Status Quo* and \$960,595 for *Expansion*. The cost of *Status Quo* was lower than the cost of *No Intervention*, indicating that the present provision of IBI was preferable to providing no IBI at all. While significant costs were incurred under all scenarios, the cost of *Expansion* was lowest, resulting in savings of \$34,479 per individual over his or her lifetime compared to *Status Quo*. Expansion of the current program to fund IBI for all autistic children (n = 1,309) in Ontario younger than six years of age results in net cost savings of \$45,133,011 for the government. The greatest number of dependency-free life years was gained under *Expansion*: 4.5 years per person compared to *No Intervention* and 2.8 years per person compared to *Status Quo*. *Expansion* is the dominant strategy, as it yields both a decrease in cost as well as gains in dependency-free years.

TABLE 2. Average costs per person of *No Intervention*, *Status Quo* and *Expansion* and cost savings from pair-wise comparisons

AGE RANGE	COST ITEM	NORMAL		SEMI-DEPENDENT		VERY DEPENDENT	
		ANNUAL COST (\$)	PV OF TOTAL COST (2003 \$)	ANNUAL COST (\$)	PV OF TOTAL COST (2003 \$)	ANNUAL COST (\$)	PV OF TOTAL COST (2003 \$)
Intervention Age (2-5)	IBI and other costs: <i>No Intervention</i>	5,378	15,211	5,378	15,211	5,378	15,211
	OR						
	IBI and other costs: <i>Status quo</i> *	33,414	94,516	33,414	94,516	33,414	94,516
	OR						
	IBI and other costs: <i>Expansion</i>	81,048	229,252	81,048	229,252	81,048	229,252
Schooling Age (5-18)	Education and Respite Services	6,616	64,393	21,422	208,490	38,672	376,372

continued

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Adulthood (18-65)	Day pro- grams, residential costs, and assisted employ- ment pro- gram costs	0	0	37,380	588,568	75,648	1,191,110
Total cost	No Intervention		\$ 79,604		\$ 812,269		\$ 1,582,693
	Status quo		\$ 158,909		\$ 891,574		\$ 1,661,998
	Expansion		\$ 293,645		\$ 1,026,310		\$ 1,796,734
Average cost per individual (PV): No Intervention †			\$ 1,014,315				
Average cost per individual (PV): Status quo ‡			\$ 995,074				
Average cost per individual (PV): Expansion **			\$ 960,595				
Incremental cost savings per individual: No Intervention → Status quo			\$ 19,241				
Incremental cost savings per individual: No Intervention → Expansion			\$ 53,720				
Incremental cost savings per individual: Status quo → Expansion			\$ 34,479				
Cost savings for cohort (n=1,309): No Intervention → Status quo			\$ 25,186,469				
Cost savings for cohort (n=1,309): No Intervention → Expansion			\$ 70,319,480				
Cost savings for cohort (n=1,309): Status quo → Expansion			\$ 45,133,011				
* Based on 485 individuals out of 1,309 receiving IBI and all 1,309 receiving respite services and speech and language therapy							
† Based on a weighted average: 25% normal, 25% semi-dependent, 50% very dependent							
‡ Based on a weighted average: 26.9% normal, 34.3% semi-dependent, 38.9% very dependent							
** Based on a weighted average: 30% normal, 50% semi-dependent, 20% very dependent							

Sensitivity analyses

The cost-effectiveness model was run with productivity costs to examine the economic impact of IBI from a partial societal perspective. Inclusion of productivity costs incurred by semi- and very dependent adults resulted in increased cost savings from *Expansion* of \$54,757 per person and \$71,676,776 for the entire cohort compared to *Status Quo*.

TABLE 3. Results of sensitivity analysis varying the efficacy of IBI

		DECREASED EFFICACY	BASELINE CASE	INCREASED EFFICACY	
Efficacy rates	Normal	25.0%	30.0%	40.0%	
	Expansion	Semi-dependent	50.0%	50.0%	50.0%
		Very dependent	25.0%	20.0%	10.0%
	Status quo	Normal	25.0%	26.9%	30.6%
		Semi-dependent	34.3%	34.3%	34.3%
		Very dependent	40.7%	38.9%	35.2%
Results	Incremental savings (cost) per individual: Status quo → Expansion	\$(13,493)	\$ 84,031	\$ 128,433	
	Discounted dependency-free years gained: Status quo → Expansion	2.0 years	2.8 years	4.4 years	

IBI efficacy was modified to accommodate controversy in the research literature (Table 3). When the efficacy of IBI was increased to 40% of subjects who achieve normal functioning, 50% achieving semi-dependent functioning and 10% achieving very dependent functioning, the cost savings from *Expansion* compared with *Status Quo* increased to \$128,433 per person. In contrast, under the assumption that IBI yielded efficacy rates of 25% for normal functioning, 50% for semi-dependent functioning and 25% for very dependent functioning, *Expansion* cost \$13,493 more per person compared to *Status Quo* and yielded gains of 2.0 dependency-free years per person. Results of the sensitivity analyses suggest that a significant drop in treatment efficacy from the base case scenario would be required in order to yield a net cost for achieving dependency-free years in this population.

Varying the discount rate modified the present value of the cost savings. With a discount rate of 1%, cost savings from *Expansion* were even greater than those realized in the base case. Cost savings were not realized with a discount rate of 5%: it cost \$29,912 more per person to expand from *Status Quo* to *Expansion*, but gains of 1.8 dependency-free years per person were still realized under *Expansion*.

Discussion

The results demonstrate that expansion of the IBI program, which currently serves 485 children (*Status Quo*), to all 1,309 autistic children in Ontario (*Expansion*) would

yield savings of \$45,133,011 over the entire cohort's lifetime (from two to 65 years of age). Significant costs are incurred under both *Status Quo* and *Expansion*; however, under *Expansion*, the government would spend \$45 million less on autistic individuals when compared with *Status Quo*.

The cost of expanding IBI to all autistic individuals is small (less than 10% of total costs) compared to the significant cost of educating and supporting semi- and very dependent individuals over their lifetime. The present value of total costs incurred during intervention (ages two to five), including respite services and speech and language therapy, is higher for *Expansion* (\$229,252 per person) compared with *Status Quo* (\$94,516 per person). However, the larger intervention cost under *Expansion* yields lower support costs during schooling and adulthood (ages five to 65) compared to *Status Quo*. The primary reason for cost savings from expansion of IBI, from *No Intervention* to *Status Quo* and from *Status Quo* to *Expansion*, is the change in the distribution of functional dependence. Increased provision of IBI results in a shift of individuals from the very dependent to semi-dependent category and, to a lesser extent, from the semi-dependent to the normal-functioning group.

To guard against criticisms of previous economic evaluations (Marcus et al. 2000), IBI efficacy rates in this study were deliberately conservative. The proportion of children who attain normal functioning from IBI was set lower, and the proportion of children who function normally without IBI was set higher, than the proportions cited in the literature (Jacobson et al. 1998; Hildebrand 1999). As a result, cost savings realized under this model (\$34,479 per individual for *Expansion* vs. *Status Quo* and \$53,720 per individual for *Expansion* vs. *No Intervention*) are lower than those reported by previous studies (Jacobson et al. 1998; Hildebrand 1999). Lower normalization rates under *No Intervention* and higher normalization rates from IBI would yield more favourable results for expansion of the current IBI program in Ontario.

Although the costing data utilized in this study are specific to Ontario, our findings may be generalized to inform health policy decisions in other jurisdictions. The increased awareness of intensive behavioural intervention and its high program cost have made the financing of IBI and its cost-effectiveness relevant concerns for governments and other payer organizations. The grounding of our model parameters in peer-reviewed research evidence and the scope of the sensitivity analyses make our findings relevant for policy decision-makers.

Limitations

Several study limitations should be noted. First, only costs borne by the Ontario government were included in this economic evaluation; hence, costs borne by other payers, including autistic individuals, their families and employers, were not considered. Inclusion of such cost items as opportunity costs, quality of life of families and unpaid

caregiver expenses could potentially increase the savings realized under *Expansion* (Curran et al. 2001; Jarbrink and Knapp 2001; Jarbrink et al. 2003). Second, expansion of the IBI program may result in higher average costs per child in the short term due to shortage of qualified IBI therapists in the province and the resulting increase in their earnings. Third, this model assumed that all children initiated IBI at the age of two. However, children may be diagnosed with autism at later ages. Because of age restrictions currently enforced by the Ontario government, these children may not receive IBI for the full three-year period. This contingency may affect the efficacy of the treatment and the associated IBI costs incurred. Fourth, the 485 children currently receiving government-funded IBI in Ontario were assumed to be representative of the entire cohort of autistic children. Fifth, while healthcare utilization might be related to the level of functioning, we did not have access to such data and, hence, the cost-effectiveness analysis does not include these costs. However, since utilization may increase with the level of dependence, the cost savings identified in this study would increase if healthcare utilization were included. Sixth, the provincial government provided only aggregate costs for its entire IBI program, resulting in the very high annual IBI therapy cost of \$75,670 per child. This figure includes the operating costs associated with the launch of the IBI program in Ontario, including a large training component for new IBI therapists. As a result, costs per child are expected to decrease in coming years as start-up costs diminish. Finally, every attempt was made to obtain accurate costing information. However, in the absence of reliable estimates, costs from other jurisdictions within Canada, and costs for developmentally disabled people in general, were used to represent costs incurred for autistic individuals in Ontario.

Conclusion

This economic evaluation demonstrates positive outcomes from expansion of the current IBI program offered by the Ontario government. In the absence of high-quality evidence on the efficacy of IBI, but under reasonable assumptions, estimated cost savings in present-value terms associated with this expansion were \$45 million for the government, with potential improvement in the quality of life of autistic individuals and their families because of increased dependency-free years gained under *Expansion*. These cost savings and improvements in outcomes were largely maintained in the sensitivity analyses. However, savings to government disappeared when the annual discount rate of 5% was used or when IBI was assumed to be less effective than in the base case scenario, with *Expansion* resulting in 25%, 50% and 25% of individuals in normal, semi-dependent and very dependent categories (compared to 30%, 50% and 20% in the base case), respectively. Owing to uncertainty surrounding the efficacy of IBI, further study in the area is recommended, perhaps in the form of a randomized, controlled trial, to allow more definitive economic evaluations in the future.

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Correspondence may be directed to: Dr. Peter C. Coyte, Department of Health Policy, Management and Evaluation, Faculty of Medicine, University of Toronto, 155 College Street, Suite 425, Toronto, ON M5T 3M6; tel.: 416-978-8369; fax 416-978-7350; email: peter.coyte@utoronto.ca.

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**Washington State
Health Care Authority**

Agency Medical Director Comments

**Health Technology Clinical Committee
ABA Therapy for Autism**

Background: Autism Spectrum Disorders (ASD) are common neurodevelopmental disorders with:

- Significant impairments in social interaction, behavior, communication impaired cognitive skills and sensory perception.
- Symptoms and treatments that vary widely including a range of behavioral, psychosocial, educational, and medical problems
- Goals for treatment that vary for different children with emphasis on communication, social interactions, and developing greater functional skills & independence
- Important questions remaining about the efficacy of therapies (Applied Behavioral Analysis) and which individuals with ASD benefit

Estimated WA State Insured Autism Population Estimate, Ages 2-12

	2006	2007	2008	2009
Total DSHS 2-12 yrs	333,592	336,949	358,745	399,124
Total UMP/PEP 2-12 yrs	16,675	17,303	21,903	22,450
Total Children 2-12 yrs	350,267	354,202	380,648	421,574
Estimate Autism (@1/110)	3,184	3,220	3,460	3,832

Prevalence of Autism Spectrum Disorders ---Autism and Developmental Disabilities Monitoring Network, United States, 2006

Abstract Problem/Condition: Autism spectrum disorders (ASDs) are a group of developmental disabilities characterized by atypical development in socialization, communication, and behavior.

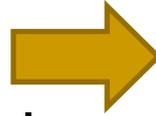
Interpretation:

- In 2006, on average, approximately 1% or one child in every 110 in the 11 ADDM sites was classified as having an ASD (approximate range: 1:80--1:240 children [males: 1:70; females: 1:315]).
- The average prevalence of ASDs identified among children aged 8 years increased 57% in 10 sites from the 2002 to the 2006 ADDM surveillance year.
- Although improved ascertainment accounts for some of the prevalence increases documented in the ADDM sites, a true increase in the risk for children to develop ASD symptoms cannot be ruled out.
- On average, although delays in identification persisted, ASDs were being diagnosed by community professionals at earlier ages in 2006 than in 2002.

Early intensive behavioral and developmental interventions vs. Typical Benefits

Applied behavior analysis (ABA)

- A broad category of early intensive behavioral and developmental interventions;
- Tx focused on associated behaviors and other behavioral interventions assessing a variety of targets



Our AMGD recommendations will address:

1. Approaches aimed at core symptoms: Early intensive behavioral and developmental approaches

We will not be discussing

2. Approaches aimed at core symptoms: Social skills approach
3. Approaches aimed at core symptoms: Play-/Interaction-based approach
4. Approaches aimed at commonly associated symptoms / Additional approaches

Side by Side Treatment and Coverage

ABA types of Therapies

- Floor time, the Social Communication Emotional Regulation Transactional Support model
- Early Start Denver Model [ESDM]
- UCLA/Lovaas model and the ESDM
- Pivotal Response Training,
- Hanen More than Words,
- Social pragmatic intervention

Current UMP, DSHS, Medicaid Coverage

- Psychotherapy services
- Physical therapy
- Occupational therapy
- Speech therapy
- Pharmaceutical drugs
- Local school districts
- **EPSDT (0-21) and Neuro-developmental Centers of Washington (0-6)**

State Agencies Policies

Coverage:

- No state agencies cover ABA therapy

•Licensure

- ABA therapists are not licensed in WA State
(extensive variation in training and credentialing)

Cost and Utilization of Treatments with the ICD9 for Autism in Medicaid (2006-2009)

CLAIM TYPE	Paid	Service #	Clients #
EPSDT Claim	\$ 22,626	262	259
Home Health Claim	\$ 716	1	1
Inpatient Claim	\$ 1,755,320	193	186
Med Vendor Claim	\$ 159,798	1050	799
OPPS Claim	\$ 520,742	3113	2582
Outpatient Claim	\$ 564,770	2051	1656
Outpatient Claim	\$ 30	1	1
Professional Claim	\$ 258,414	3257	2390
Grand Total	\$ 3,282,416	9928	7874

ICD9 for Autism, counts unique clients/month

State Mental Health Services for Children

- **Uniform Medical Plan, Public Employee Benefit Plan, 2011**
 - Outpatient mental health and behavioral health therapies, unlimited.
 - Outpatient physical, occupational, and speech therapy services up to 60 visits per calendar year
 - Inpatient/outpatient neurodevelopmental therapy, up to 60 visits per calendar year.
 - Drugs on the preferred drug list, including stimulants, antidepressants, mood stabilizers, anti-anxiety and anti-psychotics.

State Mental Health Services for Children

DSHS/Medicaid, 2011

- Psychotherapy services provided by psychiatrists, psychiatric ARNPs, psychologists, and licensed mental health professionals
 - insight oriented, behavior modifying/supportive, individual, family or group, up to 20 hours per year
 - cognitive behavioral therapy
 - elements of other therapies as needed
 - sensory integration,
 - verbal behavior intervention
 - applied behavioral analysis.
- Physical therapy (PT)
 - therapies to develop strength, endurance, range of motion, and flexibility;
 - re-education of movement, balance, coordination, kinesthetic sense, posture, proprioception for sitting, gait training;
 - group therapy;
 - dynamic activities to improve functional performance.

State Mental Health Services for Children

DSHS/Medicaid, 2011, cont.

- Occupational therapy (OT)
 - development of cognitive skills to improve attention, memory, problem solving
 - enhancement of sensory processing and adaptive responses to environmental demands
 - self-care/home management in activities of daily living
 - use of assistive/adaptive equipment
 - community and work integration training
 - Speech therapy (ST)
 - treatment and evaluation of speech, language, voice communication and auditory processing,
 - speech-generating devices,
 - oral and pharyngeal swallowing function.
 - Annual coverage for outpatient PT, OT and ST is not limited for clients 20 years old and younger, daily coverage is limited to one treatment unit per day.
 - Pharmaceutical drugs for behavioral management.
-

Multiple Agency Fiscal Note Summary

Bill Number: 5059 SB

Title: Autism spectrum disorders

II. A - Brief Description Of What The Measure Does That Has Fiscal Impact

This bill directs health plans to provide care and treatment of autism spectrum disorders. The mandate would apply to most health plans regulated by Title 48 RCW and self-insured medical plans. It does not apply to Basic Health or Medicaid. It adds new substantively identical sections to chapters 41.05 RCW and 48.43 RCW. This bill impacts the Public Employees Benefits Board's (PEBB) Insured and Self-Insured medical plans.

Part III: Expenditure Detail

III. A - Expenditures by Object Or Purpose

	FY 2012	FY 2013	2011-13	2013-15	2015-17
FTE Staff Years					
N-Grants, Benefits & Client Services	46,315,652	95,981,177	142,296,829	213,336,634	245,509,597
Total:	\$46,315,652	\$95,981,177	\$142,296,829	\$213,336,634	\$245,509,597

AMDG EVIDENCE FOCUS:

BEHAVIORAL INTERVENTIONS THAT UTILIZE ABA

Overall Evidence of Effectiveness for any outcome:

- All behavioral interventions using ABA, other than UCLA/Lovaas - Insufficient SOE
- The EIBDI intervention using UCLA/Lovaas SOE is Low. Low confidence that the evidence reflects the true effect. Further research is likely to change confidence in the estimate of effect and is also likely to change the estimate.
 - Data from which any evidence based conclusions can be drawn are limited to 6 studies rated fair quality of a total of 272 preschool age children; with varied diagnostic criteria; and with varied treatment type and intensity ranging from 10-40 hours.

Low Evidence, Limited on Health Outcomes (AHRQ Appendix I)

- “Studies commonly assessed IQ, language and **Adaptive behavior** outcomes
 - Evidence “suggests that certain cognitive/language and educational gains may be durable.
 - “It is less clear that adaptive behavior skills see similar patterns of improvement.”
-

AMDG EVIDENCE FOCUS:

BEHAVIORAL INTERVENTIONS THAT UTILIZE ABA

- Most of the reviews generally concluded that the evidence base for EIBI is inadequate, (AHRQ APPENDIX G. Discussion of Recent Systematic Reviews of Therapies for Children with ASDs) noting:
 - variability in treatment and intervention,
 - limited follow-up,
 - lack of comparative studies,
 - need for replication, and unclear inclusion and exclusion criteria
 - Three meta-analyses found an average large effect size for IQ change Eldevik; Reichow and Wolery but noted significant concerns about the included studies, such as limited accounting for the effects of maturity, lack of equivalent groups, uncertain treatment fidelity, and small sample sizes. Several authors also noted the need for studies comparing EIBI to other approaches that have been similarly empirically tested.
- Other areas for improvement noted included a need for larger sample sizes; longer follow-up to allow for evaluation of the durability of effects; greater treatment fidelity; improved reporting of methodological and participant characteristics; and greater consistency in treatment approaches and outcomes measurement.

AMDG: General Concerns related to the Evidence

- **Randomized, controlled trials:**
 - Over all the level of evidence is fair to poor
 - Are limited in number and differential in the types of interventions
 - Minimal head to head studies - Many of the studies were randomized with waiting lists rather than comparative therapies
- **Sample sizes:**
 - Most studies had limited numbers (despite a purported prevalence of 1/150)
- **Outcomes:**
 - The types of treatment vary greatly, both within and across the available studies, especially for the control groups
 - No clear threshold for clinically meaningful improvement in outcome measures
- **Longer follow-up needed:**
 - Autism is a chronic condition yet half of the studies followed children for approximately 2 years or less, and some for only 1 year.
 - This is not sufficient follow-up time to assess the potential impact of an intervention over a lifetime.
- **Incremental research strategy needed:**
 - Outcomes are varied and used intermediate rather than functional outcomes
 - Many outcomes relied on parental reports vs. functional changes (e.g. school attendance)

AMDG & AGENCIES SUMMARY

Safety

- No direct concerns related to any of the ABA therapies.
 - No indirect concerns related to use of current alternatives including medications (WA state is one of least reliant on mental health drugs)

Effectiveness

- Evidence is at best low from weak RCTs with the following methodological flaws:
 - probable bias in non-blinded outcome reporting
 - no RCTs with head to head comparison with usual care

Cost

- Cost-effectiveness unknown, but direct cost is substantial
 - The SB5059 fiscal note costs were assumed to be \$29K/client (\$213 Million 2013-15)
-

AMDG Recommendations

- The science for ABA types does not have a sufficient base to justify a \$29-50K benefit
- The AMDG do not recommend ABA type of therapies as a covered benefit
- The AMDG do recommend better promotion of existing benefits to pediatric and PCP

ABA Therapy for Autism

Questions



Applied Behavioral Analysis and Other Behavioral Therapies for the Treatment of Autism Spectrum Disorder

Presented by: Alison Little, MD, MPH
Center for Evidence-based Policy
Date: June 17, 2011

Introduction

- Background
- Methods
- Key Questions
- Findings
- Guidelines
- Policy Considerations
- Summary

Background - General

- Autism spectrum disorders (ASD) are a group of pervasive developmental disorders characterized by impairments in communication, behavior and social interaction, and by repetitive patterns of behaviors and interests (Warren et al., 2011)
- Prevalence is estimated to be 1 in 110 children, with variation across states, and cost is estimated to be \$25 billion dollars per year (CDC, 2002)
 - About a third of individuals with ASD have epilepsy and three quarters have mental retardation
- Expression and severity of symptoms of ASDs differ widely
- Treatments of ASD and treatments of symptoms commonly associated with ASD (e.g., anxiety, sensory difficulties) include a range of behavioral, psychosocial, educational, medical, and complementary approaches that vary by a child's age and developmental status

Background – Behavioral Interventions

- There is no universal consensus regarding which treatment interventions are most effective.
- ASD currently lacks a curative treatment.
- The goals of treatment for ASDs focus on improving core deficits in communication, social interactions, or restricted behaviors, as changing these fundamental deficits may help children develop greater functional skills and independence.

Background – Behavioral Interventions

- Common behavioral treatment strategies are
 - based on learning theory
 - use reinforcement, promoting, and shaping techniques to increase positive behaviors and decrease frequency of negative behaviors
- Behavioral treatments for ASD work to build communication, play, social, academic, self-care, work and community living skills
- Therapies often adapted to include parents and teachers as part of therapy

Background – Behavioral Interventions

Behavioral interventions for ASD include:

- Early Intensive Behavioral and Developmental interventions
- Social skills interventions
- Play/interaction-based interventions
- Cognitive Behavioral Therapy
- Neurofeedback
- Sleep interventions

Background – Behavioral Interventions

Some of these utilize principles of Applied Behavioral Analysis (ABA):

- Umbrella term used to describe the principles and techniques used in the assessment, treatment and prevention of challenging behaviors and the promotion of new desired behaviors
- Goal is to teach new skills and reduce challenging behaviors using systematic reinforcement

This presentation will focus only on those behavioral therapies that utilize principles of ABA.

Background – Behavioral Interventions

- Early Intensive Behavioral/Developmental Interventions (EIBDI): prescriptive, high intensity, therapy may include up to 40 hr/wk for 2 yrs or more, intervention based in ABA theory. Interventions grouped into three categories:
 - Manualized programs
 - ✓ UCLA / Lovaas model (one-on-one therapy sessions and Discrete Trial Teaching)
 - ✓ Early Start Denver Model (ESDM) (ABA principles and relationship-based approaches in young children)
 - Other intensive treatment approaches that have not been manualized
 - Interventions focused on key pivotal behaviors using parent training (e.g., Pivotal Response Training, Hanen More than Words)

Background – Behavioral Interventions

The only other behavioral intervention that utilizes the principles of ABA to some degree are play- and interaction-based interventions

- Focus on using interactions between children and adults
- Target skills including joint attention and play abilities
 - Stepping Stones Triple P Program
 - Relationship Development Intervention program
 - Mifne model
 - Floortime model
 - Others

Methods

- For the WA HTA program, the AHRQ systematic review, *Comparative Effectiveness of Therapies for Children with Autism Spectrum Disorders*, identified as sole evidence source (Warren et al., 2011)
- Search for relevant clinical practice guidelines using Guidelines.gov database
- Quality of included systematic review and guidelines rated with standard MED instruments
- State, private payors, and policy websites searched to identify insurance coverage policies

Key Questions

- **KQ1.** Among children ages 2 to 12 with ASDs, what are the short- and long-term effects of available behavioral, medical, allied health, or CAM treatment approaches?
- **KQ2.** Among children ages 2 to 12, what are the modifiers of outcome for different treatments or approaches?
- **KQ3.** Are there any identifiable changes early in the treatment phase that predict treatment outcomes?
- **KQ4.** What is the evidence that effects measured at the end of the treatment phase predict long-term functional outcomes?

Key Questions

- **KQ5.** What is the evidence that specific intervention effects measured in the treatment context generalize to other contexts (e.g., people, places, materials)?
- **KQ6.** What evidence supports specific components of treatment as driving outcomes, either within a single treatment or across treatments?
- **KQ7.** What evidence supports the use of a specific treatment approach in children under the age of two who are at high risk of developing autism based upon behavioral, medical, or genetic risk factors?

Warren et al. (2011) Methods

- MEDLINE[®], ERIC and PsycInfo[®], grey literature, reference lists
- Included studies published between January 2000 – May 2010, in English
- Excluded:
 - Medical studies with fewer than 30 participants
 - Behavioral, education, and allied health studies with fewer than 10 participants
 - Case reports

Warren et al. (2011) Methods

- Single-subject studies excluded only if fewer than 10 participants
- Two reviewers quality assessed each study
 - Differences resolved through discussion
 - Studies rated as good, fair, or poor
- Overall strength of evidence assessed using *EPC Methods Guide for Effectiveness and Comparative Effectiveness Reviews*
 - Strength of evidence presented as insufficient, low, moderate, or high

Warren et al. (2011) Methods

- Single subjects (or N-of-1) research common study design in education and psychology fields
 - Individual serves as his/her own control
 - Intervention is repeated at different times
 - Evaluation is by blinded assessor
 - All other factors must remain constant
- Although standards for single subject research exist, they are not widely applied in research on interventions for ASD
- By design, lack external validity or generalizability

Warren et al. (2011) – Findings

Strength of Evidence

- Strength of evidence (SOE) = confidence that the observed effect is unlikely to change with future research
 - Describes the adequacy of the current research (quality and quantity). Evaluated using methods established in the EPC methods guide using 4 domains (risk of bias, consistency, directness, precision).
- No behavioral intervention had better than a low SOE
= Low confidence that the evidence reflects the true effect. Further research is likely to change confidence in the estimate of effect and is also likely to change the estimate.
- All behavioral interventions other than UCLA/Lovaas had insufficient SOE

Warren et al. (2011) Overall Conclusions

- **Results.** Of 4,120 citations, 714 required full text review and 59 unique studies were included, 13 were good quality, 56 were fair, and 90 poor (78 included for Behavioral Interventions)
- **Conclusions**
 - Medical interventions including risperidone and aripiprazole show benefit for reducing challenging behaviors in some children with ASDs, but side effects are significant.
 - Some behavioral and educational interventions that vary widely in terms of scope, target, and intensity have demonstrated effects, but the lack of consistent data limits our understanding of whether these interventions are linked to specific clinically meaningful changes in functioning.
 - The needs for continuing improvements in methodologic rigor in the field and for larger multisite studies of existing interventions are substantial. Better characterization of children in these studies to target treatment plans is imperative.

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

- Over 100 outcome measures included in report
- Outcome measures used in studies presented:

Autism Diagnostic Interview-Revised (ADI-R)
Autism Diagnostic Observation Schedule (ADOS)
Vineland Adaptive Behavior Scales (VABS)
MacArthur Communicative Developmental Inquiry (MCDI)
Parenting Stress Index (PSI)
Stanford-Binet Intelligence Scale (SBIS)
Bayley Scales of Infant Development (BSID)
Merrill-Palmer Scale of Mental Tests (MPSMT)
Reynell Developmental Language Scales (RDLS)
Achenbach Child Behavior Checklist (ACBC)
Wechsler Individualized Achievement Test (WIAT)
Gilliam Autism Rating Scale (GARS)

British Abilities Scale (BAS)
Psycho-educational Profile-Revised (PEPR)
Griffith Scale of Infant Development (GSID)
Mullen Scales of Early Learning (MSEL)
Behavior Screening Questionnaire (BSQ)
Joy and Fun Assessment (JAFA)
Questionnaire on Resources and Stress (QRS)
Parent Feelings Questionnaire (PFQ)

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

Primary Evidence Base

- EIBDI - 34 papers/ 30 study populations (11 fair/19 poor) – overall strength of evidence: Low
 - **21 Lovaas-based**
 - 7 intensive parent training approaches
 - 4 social communication skills,
 - 2 pivotal response training (PRT),
 - 2 eclectic approaches,
 - 1 PRT with other behavioral approach
- Play-/Interaction-based Interventions - 15 papers / 13 populations (3 fair, 10 poor) – overall strength of evidence: insufficient
- Other intervention types not in presentation – they did not include ABA as a primary basis; overall strength of evidence: insufficient

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

UCLA/Lovaas based approaches

Smith (2000): an attempt to replicate Lovaas' original work (N=28, mean IQ 51, mean age 36 months) (RCT, fair)

Inclusion: 18-42 mos of age, IQ 35-75, absence of major med problems

Intervention: 25 hrs/week individual treatment X 1 year, less over next 2 years

Comparator: parent training X 3-9 months

Results: (outcome measures = SBIS, BSID, MPSMT, RDLS, VABS; blinded)

- Mean IQ for treatment group increased 15 pts, no change in control (sig between groups)
- Largest gains in pervasive developmental disorder, not otherwise specified (PDD-NOS) subgroup
- No sig differences in adaptive or challenging behavior between groups

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

UCLA/Lovaas based approaches

Hayward (2009): N=46, assignment based on geography (prospective cohort study, fair)

Inclusion: age 25-42 months, no major med problems

Intervention: 37 hrs/week intensive clinic-based treatment X 1 year

Comparator: 34 hrs/week intensive parent-managed treatment X 1 year

Results: (outcome measures = BSID-R, MPSMT, RDLS, VABS; blinded)

- Overall IQ increased 16 pts, verbal IQ 10 pts in both groups
- Sig improvement in language, adaptive functioning from baseline
- No differences between groups

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

UCLA/Lovaas based approaches

Howard (2005): N=61, non-random assignment (included parent preference) (prospective cohort, fair)

Inclusion: age < 48 months, English as primary language, no major med problems, no prior treatment > 100 hrs

Intervention: 25-40 hrs/week intensive individual treatment (behavior analytic)

Comparators: 30 hrs/week eclectic intervention, 15 hrs/week public early intervention program

Results: (outcome measures = BSID, WPPSI, SBIS, MPSMT, RDLS, VABS; unblinded)

- ABA group had sig improvement in all areas assessed
- Improved IQ 41 pts (24 pts more than comparator groups)
- No sig differences in outcome between the two comparator groups

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

UCLA/Lovaas based approaches

Cohen (2006): N=42, non-random assignment (prospective cohort, fair)

Inclusion: IQ > 35, age 18-42 months, no major med problems, < 400 hrs prior treatment, parent agreement to active participation

Intervention: Lovaas-based intervention

Comparator: Unspecified community care

Results: (primary outcome measure = BSID)

- ABA group had significantly higher IQ (mean 87, gain 25) than control (mean 73, gain 14)
- Improved (not significantly) receptive language skills
- No differences in expressive language or socialization between the groups at year 3

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

UCLA/Lovaas based approaches

Zachor (2007): N=68, non-random assignment (prospective cohort, fair)

Inclusion: no major med problems (seizure disorder, hearing deficiency)

Intervention: Lovaas-based intervention, 8 hrs/day X 1 yr

Comparator: Eclectic approach, 8 hrs/day X 1 yr

Results: (primary outcome measure = ADOS; unblinded)

- Both groups had sig improvement in language/communication and social interaction from baseline, but ABA group improved sig more

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

UCLA/Lovaas based approaches

Reed (2007): N=27, assignment based on geography, groups not similar at baseline (high intensity group had higher ability/cognition, lower autism severity) (non-randomized CT, fair)

Inclusion: age 2.5-4 yrs, no other major interventions during study

Intervention: High intensity, Lovaas-based at home, ave 30 hrs/wk X 9-10 months

Comparator: Low intensity, ave 13 hrs/wk X 9-10 months

Results: (outcome measures = GARS, PEPR, BAS, VABS; unblinded)

- High intensity group had sig improvement in intellectual (cognitive) and educational function
- Low intensity group had sig improvement in educational function
- Only difference between groups was in educational function (none in autism severity, cognitive function, adaptive behavior)

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

UCLA/Lovaas based approaches

Eikeseth (2002): N=25, (prospective cohort, fair)

Inclusion: age 4-7 years, IQ \geq 50, no major medical problems, diagnosis < 6 months prior to study

Intervention: Lovaas-based intervention (school-based), minimum of 20 hours/week for 1 yr

Comparator: Intensive, eclectic special education services, minimum of 20 hours/week for 1 yr

Results: (outcome measures = IQ, language, VABS)

- Eclectic group had higher average baseline scores across most areas of measurement compared to the behavioral group
- Lovaas group demonstrated statistically significant gains
 - IQ: +17 points vs. +4 points; Language comprehension: +13 points vs. -1; Expressive language: +23 points vs. -2; VABS Composite: +11 vs. 0

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

UCLA/Lovaas based approaches

Additional Studies- Poor Quality

- 3 cohort or nonrandomized trials were poor quality and found inconsistent results
- 5 Case series had mixed results
- 6 Chart reviews

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

Intensive Parent Training Approaches

4 Studies (2 Fair/2 Poor) Overall SOE – Insufficient

Drew (2002): N=24, mean age 23 months (RCT, fair)

Inclusion: No “general developmental delay”

Intervention: Home-based parent intervention, training 3 hrs/week X 6 wks, parents to engage 30-60 min/day X 1 year

Comparator: Community-based intervention

Results: (outcome measures = MCDI, GSID, ADI, PSI; unblinded)

- No difference between groups in nonverbal IQ, autism severity, words/gestures.
- Intervention group had decrease in overall IQ

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

Intensive Parent Training Approaches

4 Studies (2 Fair/2 Poor) Overall SOE – Insufficient

Aldred (2004): N=28, median age 48-51 months (RCT, fair)

Inclusion: age 2-6 yrs, no global DD, no severe environ deprivation in infancy, English, no hearing/vision impairment, no chronic illness in parents, must have some evidence of desire to interact with adult

Intervention: Home-based parent training in social communication skills (initial workshop, monthly intervention sessions, 6 months maintenance visits over 1 year)

Comparator: Treatment as usual

Results: (outcome measures = ADI, ADOS, VABS, MCDI, PSI; blinded)

- Intervention group had sig improvement in ADOS scores and expressive vocabulary (MCDI), no sig difference in other scores

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

Intensive Parent Training Approaches

4 Studies (2 Fair/2 Poor) Overall SOE – Insufficient

Two additional poor studies:

- Green (2010,RCT) found no significant difference in teacher rating of language/ communication or in number of children experiencing diagnostic shift between groups, but rating of parent/child interaction and parent report of language/communication improved in the intervention group
- Stahmer (2001,prospective cohort) found that the intervention resulted in changed parenting techniques and perceived language gain

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

Play/Interaction-based Interventions

4 Studies (1 Fair/3 Poor) Overall SOE – Insufficient

Kasari (2006, 2008): N=58, ages 3-4 yrs (RCT, fair)

Inclusion: age < 5, accessible for follow up, no seizures or genetic syndromes, plan to remain in program at least 4 weeks

Intervention: joint attention or symbolic play

Comparator: adult directed ABA therapies

Results: (primary outcome measure = growth in expressive and receptive language; blinded)

- Both intervention groups had sig greater growth in expressive language, as well as duration and initiation of joint attention
- Growth in receptive language not affected. Symbolic play group had more growth in play level

Warren et al. (2011) – Findings

KQ 1. Effects of behavioral treatments that utilize ABA on core/common symptoms

Play/Interaction-based Interventions

4 Studies (1 Fair/3 Poor) Overall SOE – Insufficient

Additional poor quality studies:

- Whittingham (2009, RCT) found that the intervention group had statistically significant decreases in child challenging behavior scores and that outcomes were maintained at 6 months and duplicated in the wait list control
- Field (2001, RCT), Heimann (2006, RCT), Escalona (2002, RCT) all found that the imitation intervention group had greater improvements in spending more time engaged, more social interest and greater reduction in motor activity
- Gulsrud (2007, RCT) found that all groups improved, and although there were no differences in toy pointing or sharing, the intervention group showed more improvement over time

Warren et al. (2011) – Findings

KQ #2: Modifiers of treatment outcome

- This KQ attempts to explore the degree to which child characteristics, treatment factors and systems (e.g., community) influence response to treatment
- Only 2 studies were designed and powered to allow identification of true modifiers of treatment effects, and one of these was in the allied health category (not ABA-based)

Sallows (2005): N= 24, age 24-42 months, (RCT, good)

Inclusion: age 24-42 months, neurologically “normal”, mental development index of 35 or higher

Intervention: parent directed Lovaas approach (30 hrs/wk)

Comparator: clinic directed Lovaas approach (30 hrs/wk)

Results: (outcome measures: BSID, MPSMT, RDLS, VABS, WPPSI; blinded)

- Both groups had substantial gains in multiple areas
- No group differences related to IQ, language, adaptive behavior

Warren et al. (2011) – Findings

KQ #2: Modifiers of treatment outcome

Several potential correlates suggested by literature, but lack sufficient power to confirm:

- Frequency/duration/intensity of intervention
- Child characteristics – one characteristic of particular speculation is cognitive ability/IQ:
 - Several studies of UCLA/Lovaas note pretreatment IQ predicts IQ at follow up, others have found that lower IQ at onset correlates with increased change in IQ over time
 - For parent training interventions, some studies suggest that those with lower IQ benefit more, while others have found the opposite
- Family characteristics (parental perceptions/affect)

Warren et al. (2011) – Findings

KQ #3: Early Results that Predict Outcomes

Evidence sparse

- Some evidence suggests early response to Lovaas or ESDM (increase in IQ) predicts long-term change in IQ
- Other evidence suggests change in adaptive behavior occurs over longer period of time

Warren et al. (2011) – Findings

KQ #4: End of Treatment Effects that Predict Outcomes

1 Study

Kasari (2006, 2008): N= 58, ages 3-4 yrs, (RCT, fair)

Inclusion: age < 5, accessible for follow up, no seizures or genetic syndromes, plan to remain in program at least 4 weeks

Intervention: joint attention or symbolic play for 5-6 weeks, outcomes measured at 6 and 12 months

Comparator: adult directed ABA therapies

Results: (primary outcome measure = growth in expressive and receptive language; blinded)

- Greater growth in expressive language (not receptive) and initiation of joint attention over time (both intervention groups)
- More growth in play level in symbolic play group
- More use of services overall post-intervention in control group (no difference in use of speech services)

Warren et al. (2011) – Findings

KQ #5: Generalization of Treatment Effects

- For most behavioral interventions, outcomes are assessed in settings outside the treatment setting, but are generally self- or parent-reported (unblinded)
- Most participants not followed over time, so maintenance of results over time unknown

Warren et al. (2011) – Findings

KQ #6: Drivers of Treatment Effects

- No studies were identified

Warren et al. (2011) – Findings

KQ #7: Treatment Approaches for Children <2 (at risk for autism)

4 Studies (1 good, 1 fair, 2 poor)

Dawson (2010): N= 48, mean age 38 months, (RCT, good)

Intervention: ESDM

Inclusion: age < 30 months, proximity to study center, willingness to participate > 2 years, no neurodevelopmental disorder, no major sensory/motor impairment, no seizures, no major medical problems, no psychoactive medications, no history of head injury, no prenatal exposures, IQ > 35

Comparator: community-based interventions

Results: (outcome measures = MSEL, VABS; unblinded)

- At 1 year: significant greater increase in IQ, no difference in adaptive behavior
- At 2 years: significant greater increase in IQ, receptive/expressive language, adaptive behavior, no change in autism severity or repetitive behaviors

Warren et al. (2011) – Findings

KQ #7: Treatment Approaches for Children <2 (at risk for autism)

4 Studies (2 fair, 2 poor)

McConachie (2005): N= 47, mean age 34-38 months, (non-randomized CT, fair)

Inclusion: language delay and suspicion for ASD, age 24-48 months, no serious med problem, no intensive home program

Intervention: Hanen More than Words

Comparator: wait-list control

Results: (outcome measures = MCDI, ADOS, BSQ, JAFA, QRS-F, PFQ; blinded)

- Language use sig higher in intervention group, no diff in behavior issues or autism severity

Two additional case series:

Vismara (2009): prospective case series evaluating distance learning vs. live instruction for training of parents by therapists (both effective, but fidelity required supervision)

Wetherby (2006): prospective case series evaluating Early Social Interaction Project found positive impact on ASD symptoms, but lack of control group limits conclusions

Warren et al. (2011) – Findings

Strength of Evidence

Strength of evidence (SOE) = confidence that the observed effect is unlikely to change with future research

- Describes the adequacy of the current research (quality and quantity)
- No behavioral intervention had better than a low SOE

Table 1. Intervention, strength of evidence domains, and strength of evidence for key outcomes

Intervention	Domains pertaining to Strength of Evidence (SOE):				SOE
	Risk of Bias	Consistency	Directness	Precision	
Adaptive behavior					
Behavioral, UCLA/Lovaas	Medium	Consistent	Direct	Imprecise	Low
ASDs symptom severity					
Behavioral, UCLA/Lovaas	Medium	Inconsistent	Direct	Precise	Low
Language/communication					
Behavioral, UCLA/Lovaas	Medium	Consistent	Direct	Precise	Low
IQ/cognitive					
Behavioral, UCLA/Lovaas	Medium	Consistent	Direct	Precise	Low

Warren et al. (2011) – Findings

Strength of Evidence

The following interventions had an insufficient SOE for the specified outcome:

- ESDM: adaptive behavior, ASD severity, IQ, language/communication
- Play/interaction-based (imitation): social skills
- Play/interaction-based (joint attention/symbolic play): joint attention
- Play/interaction-based (joint attention/symbolic play): joint attention
- Relationship-focused (play/interaction-based): social skills
- Parent training (EIBDI): language/communication, repetitive behaviors, social skills
- Parent focused (play/interaction-based): challenging behaviors

Warren et al. (2011) – Findings

Strength of Evidence

Limitations for the evidence:

- Many studies lacked a comparison group
- Sample size was frequently insufficient to draw conclusions
- Several studies used inappropriate comparison groups to assess treatment effectiveness
- Description of the intervention is often inadequate
- Characterization of the study population was often inadequate
- Multiple disparate outcome measures, making synthesis difficult
- Many studies use change in IQ as outcome of interest, but may not be an ideal tool to assess core ASD symptoms
- Many studies presented on numerous outcomes without adjusting for multiple comparisons, may result in reporting bias
- Duration of treatment and follow up was generally short
- Few studies accounted for concomitant interventions which may confound the observed effectiveness
- A minimum clinically significant difference has not been defined

Guidelines

Four guidelines identified:

- American Academy of Pediatrics (AAP) (2007)
 - poor quality
- National Autism Center (NAC) (2009)
 - poor quality
- New Zealand Guidelines Group (NZGG) (2008)
 - fair quality
- Scottish Intercollegiate Guidelines Network (SIGN) (2007)
 - good quality

Guidelines

AAP (2007), poor quality

- Focus: to assist pediatricians in educating families
- AAP rated as poor quality because:
 - Methods unclear (no systematic search, no study selection criteria, limited information on the quality of studies)
 - Recommendations not specific or clearly described
 - Potential COI
- Only a summary of the evidence is provided
 - Primarily a description of treatment modalities and options to consider
 - Some recommendations for drug treatment

Guidelines

NAC (2009), poor quality

- Rated as poor quality because:
 - Lack of methodologic rigor
 - No clear link between evidence and recommendation
 - Recommendations not specific
 - Lack of applicability to practice
 - Potential COI
- Groups interventions into treatment categories
 - Categories represent similar treatments
 - Difficult to know exactly which interventions are included in some categories

Guidelines – NAC cont.

- Strength of evidence rated as established, emerging, unestablished, or ineffective/harmful
- Established treatments (evidence to show these treatments are effective) include:
 - Antecedent package, behavioral package, comprehensive behavioral treatment for young children, joint attention intervention, modeling, naturalistic teaching strategies, peer training package, PRT, and schedules
- Emerging Treatments (more research is needed) include:
 - Augmentative and alternative communication device, CBT, developmental relationship-based treatment, imitation-based interventions, initiation training, language training (production), language training (production and understanding), picture exchange communication system, social communication intervention, social skills package

Guidelines

NZGG (2008), fair quality

- Rated as fair quality because:
 - Involvement of funding bodies not stated
 - Recommendations not specific
 - Applicability to practice not clear
- Literature review was limited to systematic reviews (no primary studies included)
- 10 reviews of ABA identified (4 very good quality, 4 good quality, 2 fair quality)
- Of the 4 very good quality reviews, 2 found the evidence to be insufficient to make recommendations, 1 concluded that there was no clear answer regarding the most effective therapy for ASD and 1 concluded that EIBI should be the intervention of choice, but that there were substantial threats to the validity of that conclusion

Guidelines – NZGG cont.

- Behavior management techniques should be used to intervene with problem behavior following functional behavioral assessment (Grade A: a number of studies that are valid, applicable and relevant)
 - States it is beyond the scope of the guideline to provide details on how behavioral interventions are developed and implemented
- Interventions and strategies based on ABA principles should be considered for all children with ASD (Grade A)
 - ABA does not refer to one program or technique
 - Lack of knowledge about the suitability of ABA for persons with an Asperger Syndrome diagnosis and participants over 15 years

Guidelines – NZGG cont.

- EIBI should be considered for young children to improve outcomes such as cognitive ability, language skills, and adaptive behavior (Grade B: based on studies that are mostly valid, but some concerns about volume, consistency, applicability or relevance)
 - Substantial individual variability in outcomes (from very positive improvement to no effect)
 - Regular monitoring and evaluation of intervention effectiveness is crucial

Guidelines

SIGN (2007), good quality

- Rated as good quality because:
 - Rigor of development robust and clearly described for both evidence and recommendations
 - Explicit link between evidence and recommendations
 - Low risk for conflicts of interest
 - Recommendations are specific and applicable to practice

Guidelines – SIGN cont.

- Lovaas program should not be presented as an intervention that will lead to normal functioning
 - “A” recommendation: based on at least one high quality SR, MA or RCT with very low risk of bias
 - Comprehensive literature search did not find any good quality evidence for other intensive behavioral interventions
- Behavioral interventions should be considered to address a wide range of specific behaviors (aberrant behaviors, language, living skills, academic skills, social skills)
 - “B” recommendation: based on systematic review of case control or cohort studies
 - Based on one systematic review of focal* treatments for ASD
- Interventions to support communication are indicated (e.g., visual augmentation) and interventions to support social communication should be considered
 - “D” recommendation: studies with risk of bias or expert opinion

*focal not defined

Policy Considerations

- No Medicare National Coverage Determinations for the treatment of ASD identified
- Of private payors searched (BCBS, Aetna, GroupHealth), only Aetna nationally covers treatment of ASD (includes ABA)
- 27 states currently mandate insurance coverage of ASD and 15 states (as of May 9, 2011) have pending legislation that would require coverage of autism treatment

Policy Considerations

- State coverage mandates differ substantially based on:
 - Age limits
 - Maximum benefit limits
 - Covered services
 - Application of coverage mandate to all insurers or only state regulated insurance plans
 - Licensure of ABA providers

Summary

- There is a low strength of the evidence for the effectiveness of UCLA/Lovaas ABA therapy as it pertains to
 - Adaptive behavior
 - ASD symptom severity
 - IQ/ cognitive development
 - Language/ communication
- The evidence is insufficient for all other behavioral therapies
- The evidence is insufficient to answer any other questions posed in this report

Questions or comments?

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HTCC Coverage and Reimbursement Determination Analytic Tool

HTA's goal is to achieve *better health care outcomes* for enrollees and beneficiaries of state programs by paying for proven health *technologies that work*.

To find best outcomes and value for the state and the patient, the HTA program focuses on these questions:

1. Is it safe?
2. Is it effective?
3. Does it provide value (improve health outcome)?

The principles HTCC uses to review evidence and make determinations are:

Principle One: Determinations are Evidence based

HTCC requires scientific evidence that a health technology is safe, effective and cost-effective¹ as expressed by the following standards.²

- Persons will experience better health outcomes than if the health technology was not covered and that the benefits outweigh the harms.
- The HTCC emphasizes evidence that directly links the technology with health outcomes. Indirect evidence may be sufficient if it supports the principal links in the analytic framework.
- Although the HTCC acknowledges that subjective judgments do enter into the evaluation of evidence and the weighing of benefits and harms, its recommendations are not based largely on opinion.
- The HTCC is explicit about the scientific evidence relied upon for its determinations.

Principle Two: Determinations result in health benefit

The outcomes critical to HTCC in making coverage and reimbursement determinations are health benefits and harms.³

- In considering potential benefits, the HTCC focuses on absolute reductions in the risk of outcomes that people can feel or care about.
- In considering potential harms, the HTCC examines harms of all types, including physical, psychological, and non-medical harms that may occur sooner or later as a result of the use of the technology.
- Where possible, the HTCC considers the feasibility of future widespread implementation of the technology in making recommendations.
- The HTCC generally takes a population perspective in weighing the magnitude of benefits against the magnitude of harms. In some situations, it may make a determination for a technology with a large potential benefit for a small proportion of the population.
- In assessing net benefits, the HTCC subjectively estimates the indicated population's value for each benefit and harm. When the HTCC judges that the balance of benefits and harms is likely to vary substantially within the population, coverage or reimbursement determinations may be more selective based on the variation.
- The HTCC considers the economic costs of the health technology in making determinations, but costs are the lowest priority.

¹ Based on Legislative mandate: See RCW 70.14.100(2).

² The principles and standards are based on USPSTF Principles at: <http://www.ahrq.gov/clinic/ajpmsuppl/harris3.htm>

³ The principles and standards are based on USPSTF Principles at: <http://www.ahrq.gov/clinic/ajpmsuppl/harris3.htm>

Using Evidence as the basis for a Coverage Decision

Arrive at the coverage decision by identifying for Safety, Effectiveness, and Cost whether (1) evidence is available, (2) the confidence in the evidence, and (3) applicability to decision.

1. **Availability of Evidence:**

Committee members identify the factors, often referred to as outcomes of interest, that are at issue around safety, effectiveness, and cost. Those deemed key factors are ones that impact the question of whether the particular technology improves health outcomes. Committee members then identify whether and what evidence is available related to each of the key factors.

2. **Sufficiency of the Evidence:**

Committee members discuss and assess the evidence available and its relevance to the key factors by discussion of the type, quality, and relevance of the evidence⁴ using characteristics such as:

- Type of evidence as reported in the technology assessment or other evidence presented to committee (randomized trials, observational studies, case series, expert opinion);
- the amount of evidence (sparse to many number of evidence or events or individuals studied);
- consistency of evidence (results vary or largely similar);
- recency (timeliness of information);
- directness of evidence (link between technology and outcome);
- relevance of evidence (applicability to agency program and clients);
- bias (likelihood of conflict of interest or lack of safeguards).

Sufficiency or insufficiency of the evidence is a judgment of each clinical committee member and correlates closely to the GRADE confidence decision.

Not Confident	Confident
Appreciable uncertainty exists. Further information is needed or further information is likely to change confidence.	Very certain of evidentiary support. Further information is unlikely to change confidence

3. **Factors for Consideration - Importance**

At the end of discussion at vote is taken on whether sufficient evidence exists regarding the technology's safety, effectiveness, and cost. The committee must weigh the degree of importance that each particular key factor and the evidence that supports it has to the policy and coverage decision. Valuing the level of importance is factor or outcome specific but most often include, for areas of safety, effectiveness, and cost:

- risk of event occurring;
- the degree of harm associated with risk;
- the number of risks; the burden of the condition;
- burden untreated or treated with alternatives;
- the importance of the outcome (e.g. treatment prevents death vs. relief of symptom);
- the degree of effect (e.g. relief of all, none, or some symptom, duration, etc.);
- value variation based on patient preference.

⁴ Based on GRADE recommendation: <http://www.gradeworkinggroup.org/FAQ/index.htm>

Medicare Coverage and Guidelines

Organization	Date	Outcome	Evidence Cited?	Grade / Rating
CMS National Policy Decisions – WA HTA Centers for Medicare and Medicaid Services		No CMS policy		N/A
Guidelines – WA HTA Page: 55 <i>American Academy of Pediatrics (AAP)</i>	2007	The American Academy of Pediatrics (AAP) released a guideline (poor quality) in 2007 titled <i>Management of Children with Autism Spectrum Disorders</i> The AAP guideline does not provide specific recommendations for the use of ABA but it does state that “the effectiveness of ABA-based intervention in ASDs has been well documented...”	Yes	AGREE Rating: Poor
Guidelines – WA HTA Page: 57-58 <i>National Autism Center (NAC)</i>	2009	The National Autism Center (NAC) guideline is rated poor quality; it does not give specific recommendations for interventions. The guideline groups interventions into treatment categories that are not aligned with the AHRQ report, but there is overlap with some interventions in the AHRQ report, some of which appear in the “established treatments, and others in the “emerging treatments”.	Yes	AGREE Rating: Poor
Guidelines – WA HTA Page: 58-59 <i>New Zealand Guideline Group (NZGG)</i>	2008 Supplement related to ABA Therapy in 2010	The <i>New Zealand Autism Spectrum Disorder Guideline</i> is rated as fair quality, recommends: <ul style="list-style-type: none"> ▪ Behavior management techniques should be used to intervene with problem behaviors following functional behavior assessment (Grade A). ▪ Interventions and strategies based on applied behavior analysis (ABA) principles should be considered for all children with ASD (Grade A). The guideline states that there is a lack of knowledge about the suitability of ABA for persons with an Asperger Syndrome diagnosis, and for participants aged 15 years or above. ▪ Early intensive behavioral intervention (EIBI) should be considered as a treatment of value for young children with ASD to improve outcomes such as cognitive ability, language skills, and adaptive behavior (Grade B). There is substantial individual variability in outcomes ranging from very positive improvements, through 	Yes	AGREE Rating: Fair

Organization	Date	Outcome	Evidence Cited?	Grade / Rating
		<p>minor or minimal improvements, to no effects. Regular monitoring and evaluation of intervention effectiveness is crucial.</p>		
<p>Guidelines – WA HTA Page: 59</p> <p><i>Scottish Intercollegiate Guidelines Network (SIGN)</i></p>	<p>2007</p>	<p>SIGN recommendations for the assessment and clinical treatment of ASD.</p> <p>Recommends the use of behavioral interventions to address a wide range of specific behaviors in children and young people with ASD (level of recommendation: B) and states that the Lovaas program should not be presented as an intervention that will lead to normal functioning (level of recommendation: A). Interventions to support communication in ASD such as the use of visual augmentation are recommended (level of recommendation D) and the consideration of parent mediated intervention programs is recommended as a good practice point.</p> <p>Social communication and interaction interventions are recommended for children and young people with ASD, and specific interventions for individuals should be assessed on an individual basis (level of recommendation: D).</p> <p>SIGN notes that cognitive behavioral therapy (CBT) “has been shown to be feasible in children with ASD who have a verbal IQ of at least 69” (2007, p.18) and recommends, as a good practice point, that professional be aware that some interventions require a level of verbal and cognitive development.</p>	<p>Yes</p>	<p>AGREE Rating: Good</p>

HEALTH TECHNOLOGY EVIDENCE IDENTIFICATION

Discussion Document: What are the key factors and health outcomes and what evidence is there?

<i>ABA and Other Behavioral Therapies for the Treatment of Autism Spectrum Disorder</i>	
<p>Intervention Approaches</p> <ul style="list-style-type: none"> • Positive Behavioral Interventions and Support (PBS) • Incidental Teaching • Milieu Therapy • Verbal Behavior • Discrete Trial Training • Early intensive behavioral/ developmental interventions (EIBDI) <ul style="list-style-type: none"> ○ UCLA / Lovaas model ○ Early Start Denver Model (ESDM) • Parent training: (e.g., Pivotal Response Training, Hanen) • Social skills interventions: • Play- /interaction-based interventions • Cognitive behavioral therapy 	
<p>Treatment Factors</p> <ul style="list-style-type: none"> ▪ Type ▪ Frequency ▪ Duration ▪ Intensity 	
Safety Outcomes	Safety Evidence
Mortality	
Morbidity	
Other Adverse Events	

Efficacy – Effectiveness Outcomes	Efficacy / Effectiveness Evidence
ASD Symptom Severity	
Adaptive Behavior	
Anger/Anxiety	
Challenging Behavior	
Hyperactivity	
IQ / Cognitive Abilities	
Language/Communication	
Mood/Sensory	
Repetitive Behavior	
Sleep	
Social Skills	
Other Patient Outcomes	
Special Population / Considerations Outcomes	Special Population Evidence
Gender	
Age	
Severity or IQ at initiation	
Other:	
Cost	Cost Evidence
Cost Implications	
Direct and indirect - Short term - Long term	
Cost Effectiveness	

Clinical Committee Evidence Votes

First voting question

The HTCC has reviewed and considered the technology assessment and information provided by the administrator, reports and/or testimony from an advisory group, and submissions or comments from the public. The committee has given greatest weight to the evidence it determined, based on objective factors, to be the most valid and reliable.

Is there sufficient evidence under some or all situations that the technology is:

	Unproven (no)	Equivalent (yes)	Less (yes)	More (yes)
Effective				
Safe				
Cost-effective				

Discussion

Based on the evidence vote, the committee may be ready to take a vote on coverage or further discussion may be warranted to understand the differences of opinions or to discuss the implications of the vote on a final coverage decision.

- Evidence is insufficient to make a conclusion about whether the health technology is safe, efficacious, and cost-effective;
- Evidence is sufficient to conclude that the health technology is unsafe, ineffectual, or not cost-effective
- Evidence is sufficient to conclude that the health technology is safe, efficacious, and cost-effective for all indicated conditions;
- Evidence is sufficient to conclude that the health technology is safe, efficacious, and cost-effective for some conditions or in some situations

A straw vote may be taken to determine whether, and in what area, further discussion is necessary.

Second vote

Based on the evidence about the technologies' safety, efficacy, and cost-effectiveness, it is

_____ Not Covered. _____ Covered Unconditionally. _____ Covered Under Certain Conditions.

Discussion Item

Is the determination consistent with identified Medicare decisions and expert guidelines, and if not, what evidence is relied upon.

Clinical Committee Findings and Decisions

Next Step: Cover or No Cover

If not covered, or covered unconditionally, the Chair will instruct staff to write a proposed findings and decision document for review and final adoption at the following meeting.

Next Step: Cover with Conditions

If covered with conditions, the Committee will continue discussion.

- 1) Does the committee have enough information to identify conditions or criteria?
 - Refer to evidence identification document and discussion.
 - Chair will facilitate discussion, and if enough members agree, conditions and/or criteria will be identified and listed.
 - Chair will instruct staff to write a proposed findings and decision document for review and final adoption at next meeting.
- 2) If not enough or appropriate information, then Chair will facilitate a discussion on the following:
 - What are the known conditions/criteria and evidence state
 - What issues need to be addressed and evidence state

The chair will delegate investigation and return to group based on information and issues identified. Information known but not available or assembled can be gathered by staff ; additional clinical questions may need further research by evidence center or may need ad hoc advisory group; information on agency utilization, similar coverage decisions may need agency or other health plan input; information on current practice in community or beneficiary preference may need further public input. Delegation should include specific instructions on the task, assignment or issue; include a time frame; provide direction on membership or input if a group is to be convened.

Efficacy Considerations:

- What is the evidence that use of the technology results in more beneficial, important health outcomes? Consider:
 - Direct outcome or surrogate measure
 - Short term or long term effect
 - Magnitude of effect
 - Impact on pain, functional restoration, quality of life
 - Disease management
- What is the evidence confirming that use of the technology results in a more beneficial outcome, compared to no treatment or placebo treatment?
- What is the evidence confirming that use of the technology results in a more beneficial outcome, compared to alternative treatment?
- What is the evidence of the magnitude of the benefit or the incremental value
- Does the scientific evidence confirm that use of the technology can effectively replace other technologies or is this additive?
- For diagnostic tests, what is the evidence of a diagnostic tests' accuracy
 - Does the use of the technology more accurately identify both those with the condition being evaluated and those without the condition being evaluated?
- Does the use of the technology result in better sensitivity and better specificity?
- Is there a tradeoff in sensitivity and specificity that on balance the diagnostic technology is thought to be more accurate than current diagnostic testing?
- Does use of the test change treatment choices

Safety

- What is the evidence of the effect of using the technology on significant morbidity?
 - Frequent adverse effect on health, but unlikely to result in lasting harm or be life-threatening, or;
 - Adverse effect on health that can result in lasting harm or can be life-threatening.
- Other morbidity concerns
- Short term or direct complication versus long term complications
- What is the evidence of using the technology on mortality – does it result in fewer adverse non-fatal outcomes?

Cost Impact

- Do the cost analyses show that use of the new technology will result in costs that are greater, equivalent or lower than management without use of the technology?

Overall

- What is the evidence about alternatives and comparisons to the alternatives
- Does scientific evidence confirm that use of the technology results in better health outcomes than management without use of the technology?

