

Translational Research in Autism: A national perspective

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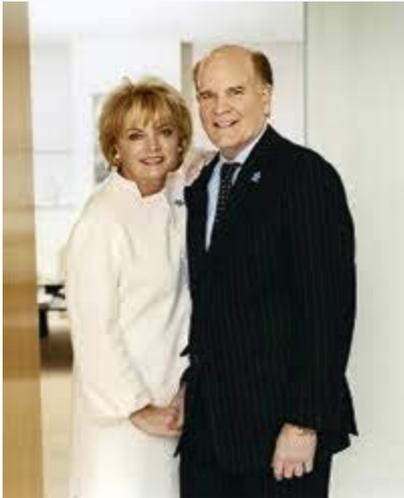
Sr. Director of Clinical and Environmental Sciences

Overview of presentation



- Translational research in autism – skip through
- Mechanisms of support
 - Outside LLC (Delsia)
 - ATN
 - Investigator-Initiated awards

A little about Autism Speaks



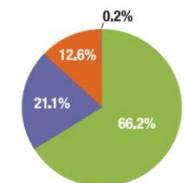
- Expanded from \$11M (2005) to \$58M (2012)
- 2013 - \$24M research funding
 - Grants, initiatives, resources,

2012 Financial Snapshot

Funding Sources

| | |
|---|----------|
| Walk Program/Retail Partners | \$38.9 M |
| Major Gifts, Grants and Other Contributions | \$12.1 M |
| Events | \$7.4 M |
| Interest Income and Other | \$0.1 M |

Total \$58.8 M



Translational Research

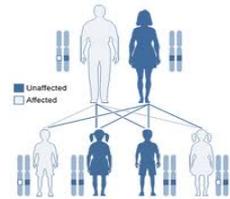
What is it, and why is it critical to our mission?

AUTISM SPEAKS™

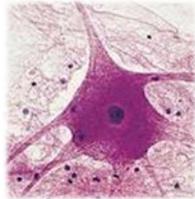
trans-lation: (n) The process of converting scientific discovery into applications that provide real-life benefit for individuals living with ASD.

Bench

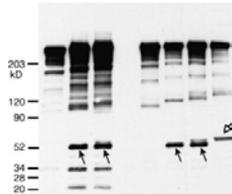
Genetics



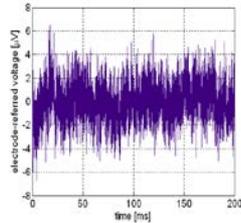
Cell Biology



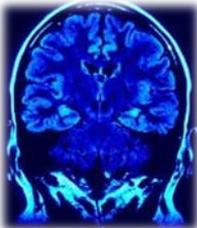
Biochemistry



Physiology



Imaging



Behavior



Development



Bedside



Medicines

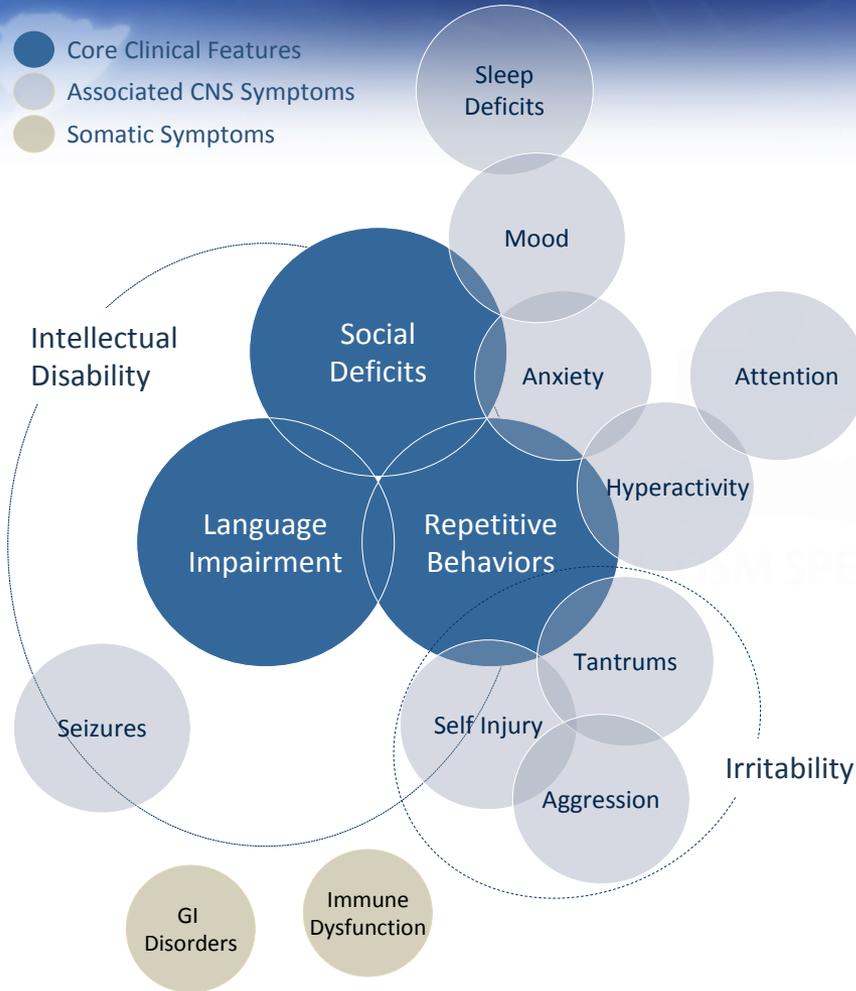


Devices



Diagnostics





Bedside



Medicines



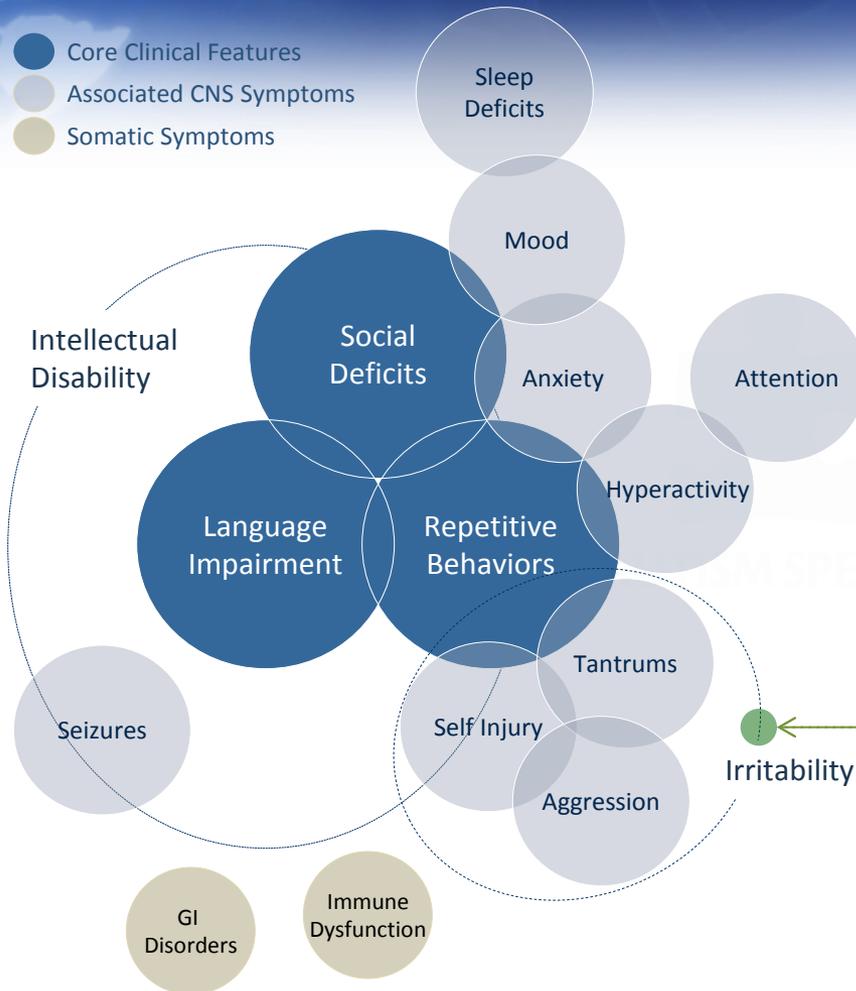
Devices



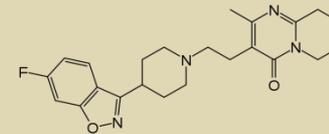
Diagnostics



AUTISM SPEAKS™ SCIENCE

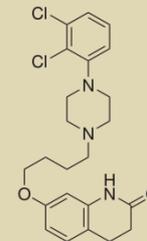


Risperdal
RISPERIDONE



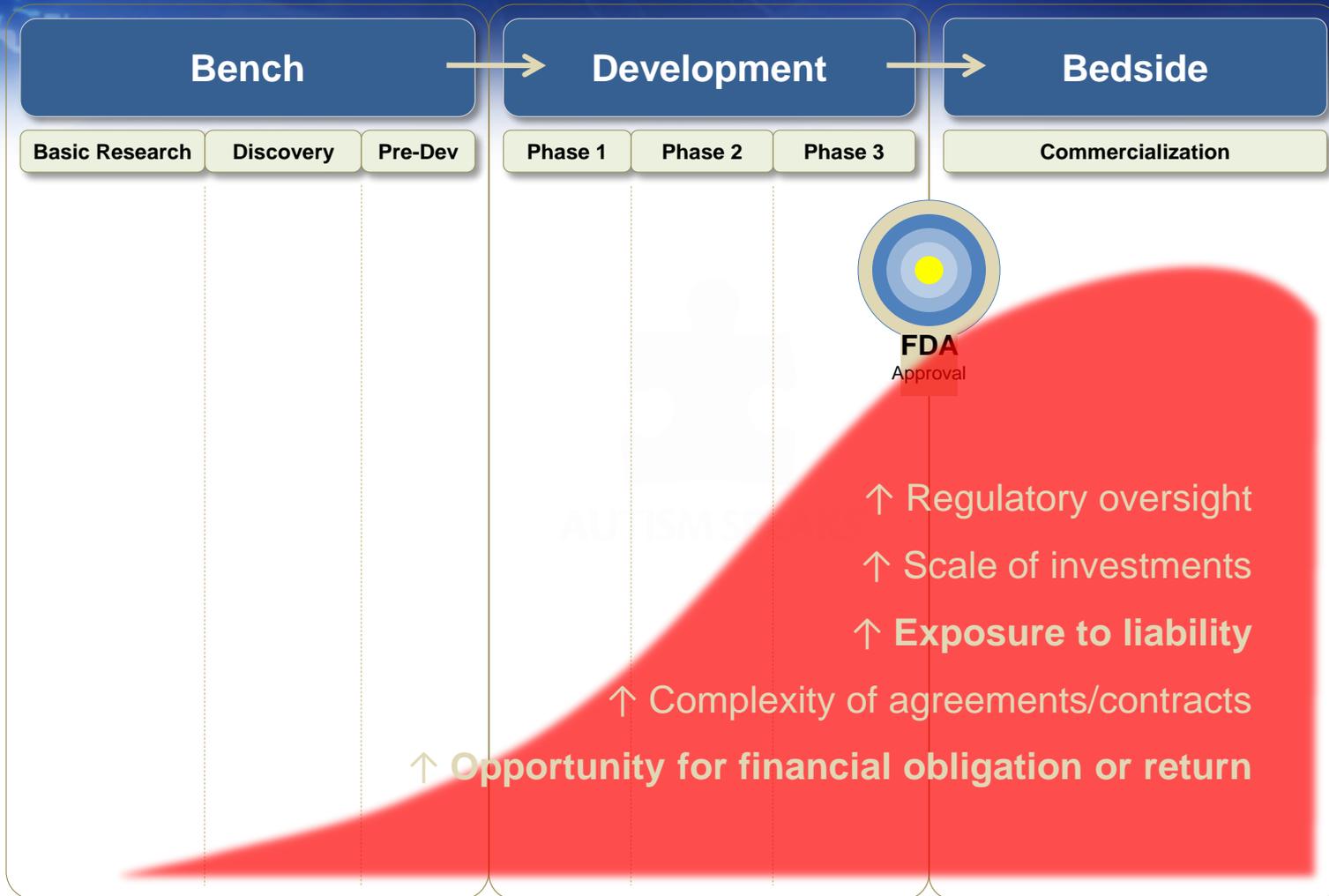
Approved by FDA: 2006
For use in children ages 5 to 16

ABILIFY
(aripiprazole)
TABLETS and ORAL SOLUTION 1 mg/mL



Approved by FDA: 2009
For use in children ages 6 to 17

Understanding the Translational Research Space



Potential approaches

- Investing in business ventures to accelerate science
- Supporting academics targeted on “bench to bedside research”
- Creating infrastructure and a platform for clinical research



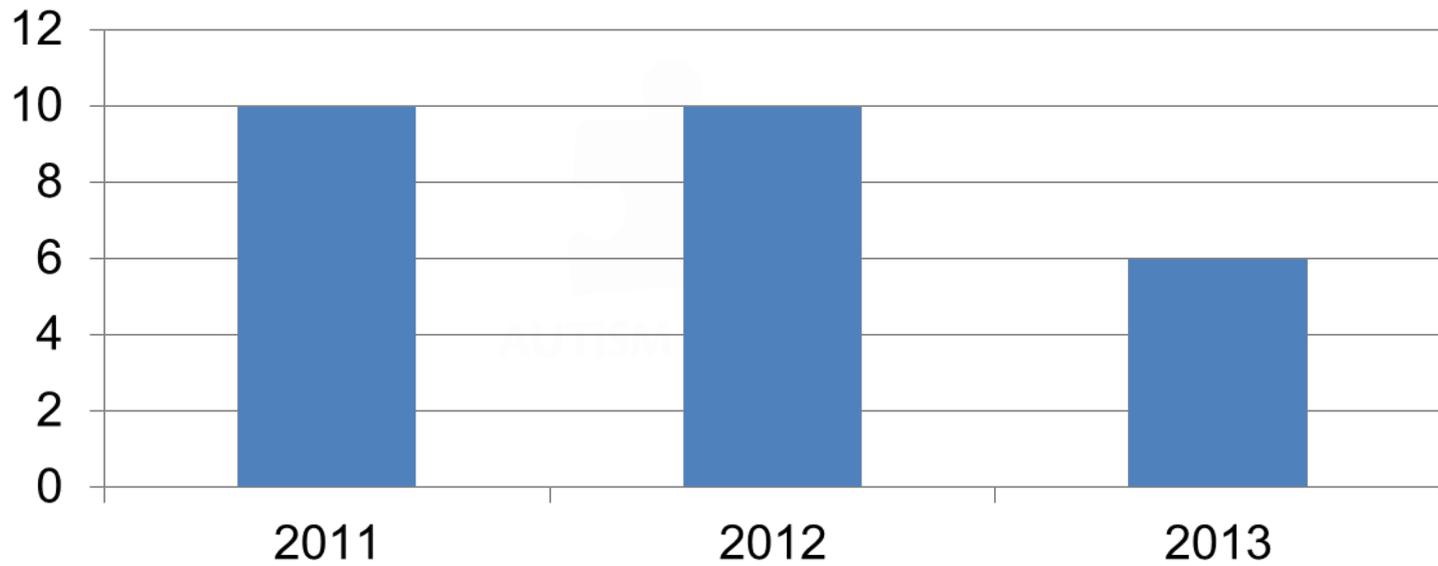
Investigator Initiated Awards



Postdoctoral awards - 4 main areas:

1. **Biomarker Discovery:**
2. **Outcome Measure Development:**
3. **Preclinical Target Validation:**
4. **Experimental Therapeutics:**

Number awarded each year



Examples



- Small molecule compounds to “wake up” the dormant paternal allele in Angelman Syndrome mice



- RCT of oxytocin



- Autism in a dish: inserting genetic mutations into human cell progenitors

Integration with other translational research programs:



- Understanding the etiological significance of attentional disengagement in infants at-risk for ASD



Challenges for patients



- Many children with autism cannot describe pain or may show discomfort behaviorally
- Disruptive behaviors make careful and thorough examination for medical issues difficult
- Failure to accurately diagnose underlying medical conditions could likely lead to more serious health problems
- Doctors have attributed medical symptoms to the individual's autism
- Parents were traveling all over the country to piece together a medical team to address their child's needs

A Model for Comprehensive Care

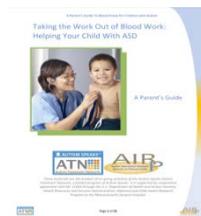
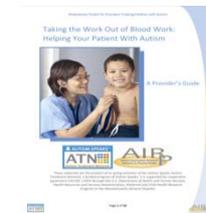
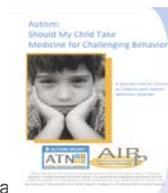
Autism Speaks Autism Treatment Network



- 17 children's hospitals with teams of doctors and specialists who understand autism and who work together across several subspecialty areas to treat the medical issues.
- Each center has a team that includes pediatricians, psychologists, sleep medicine, GI doctors, psychiatrists, genetics/metabolics and neurology
- Support a comprehensive integrated model of care focusing on the whole child
- Family-Centered
- Address the medical issues that individuals with autism face
- Develop guidelines for associated medical conditions
- Raised the bar among the medical community to recognize the importance of medical issues in autism



- 34,000 children served **annually**
- > 6300 patients in registry
- **NIMH-funded bio-repository** (473 individuals)
- Serving as the **HRSA-funded Autism Intervention Research Network for Physical Care (AIRP)**
- Over 18 toolkits and resources for families and physicians released
- 24 **peer-reviewed** papers
- **First published evidence-based guidelines** for medical treatment of GI, sleep, and ADHD (*Pediatrics*, 2012)



Tool Kits and Resources



| ATN/AIR-P Tool Kit | Number of Downloads | Month of Release |
|--|----------------------------|-------------------------|
| Constipation Guide for Parents | 506 | 2013 - July |
| Safe Medication Use | 3,404 | 2013 - June |
| Sleep Quick Tips for Parents | 293 | 2013 - February |
| Applied Behavioral Analysis | 11,122 | 2012 - June |
| Introduction to Behavioral Health Treatments | 5,922 | 2012 - June |
| Toileting | 8,343 | 2012 - June |
| Dental Provider's Guide | 3,474 | 2012 - February |
| Sleep Parent Booklet | 8,362 | 2012 - February |
| Visual Supports | 15,003 | 2011 - November |
| Blood Draws – Guide for Parents | 3,210 | 2011 - September |
| Blood Draws – Guide for Providers | 1,606 | 2011 - September |
| Medication Decision Aid | 7,910 | 2011 - September |

| ATN/AIR-P Tool Kit | ETA |
|---|----------------|
| Delivering Feedback Video for Professionals | August 2013 |
| Feeding Guide for Parents | September 2013 |
| PICA Guide for Parents | Fall 2013 |
| Vision Exam Video for Professionals | Fall 2013 |

Moving forward – what will the ATN look like in the future?

New funding opportunity/ expanding the resource

- **Expand access to the ATN Care Model** for the increasing numbers of children and youth with ASD
- **Expand care** through collaborative partnerships with community-based **provider networks**.
- Enhance the care model by **strengthening family support, enhancing training** for healthcare professionals, and incorporating **adult transition**.
- **Provide greater integration** with Autism Speaks local chapters, advocacy and awareness activities, and fundraising efforts (ex. Light It Up Blue).
- Continue to **support research** through public and private partnerships (i.e. AIR-P).

Bottom Line – network model to accelerate development

- The ATN is a **live captive audience** for translational research
 - Well characterized, standardized protocols
 - Willing to participate in additional studies
- It can leverage on existing resources, infrastructure and funding
 - Ready made clinical trial network
 - AS contribution per site modest (\$140k per year per site)
- Simultaneous functions: resources, research and recruitment