Translational Medicine Research in Autism: Challenges and Opportunities

January 25-27, 2011
Santa Monica, CA

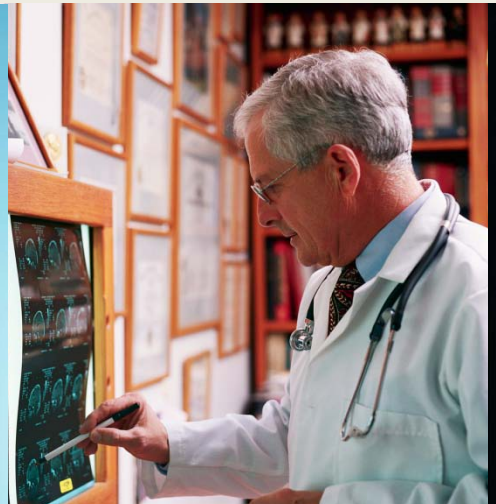
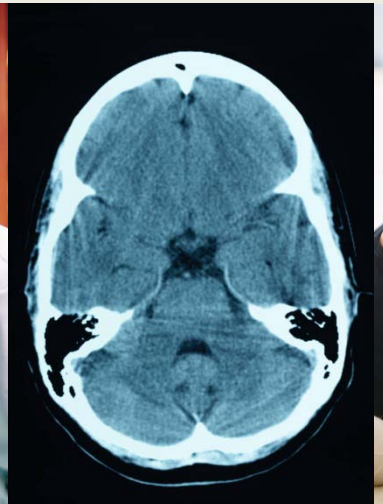
Autism Speaks Translational Medicine Research Initiative

Translational Medicine Research in Autism

Purpose of the meeting

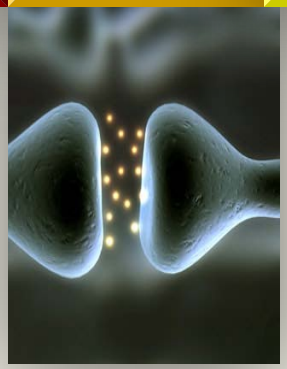
Identify strategies to accelerate the development of novel diagnostics and drugs that can assist in early detection and ameliorate the core or associated symptoms of ASD, improve response to behavioral interventions, and ultimately enhance the quality of life for people with ASD and their families

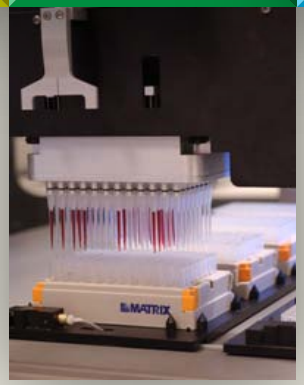



Challenging pathway to drug discovery

Autism → **Target ID** → **Assay Development** → **High Throughput screening** → **Preclinical** → **Phase I-III trials** → **FDA approval**



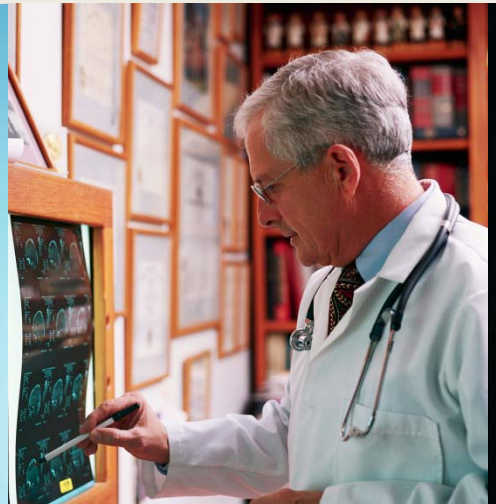
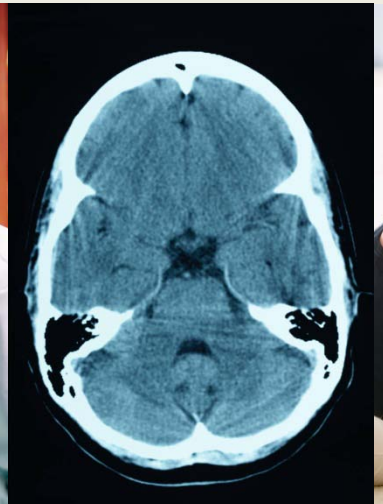


How can we accelerate discovery and development?

Objectives of meeting

1. Promote collaboration and cross-fertilization among academic community, industry, NIH, non-profit organizations, and stakeholders
2. Facilitate entry of pharmaceutical companies into the autism field
3. Identify promising drug targets and strategies for their validation
4. Identify gaps and opportunities for future research and strategies for accelerating progress




Day 1: Topics and Speakers

Translational medicine research in ASD: State of the science and pathway forward

Ed Scolnick, MD	Broad	Future of Diagnostic Tools and Treatment of Psychiatric Conditions
Thomas Insel, MD	NIMH	ASD translational research: challenges and opportunities
David Bredt, MD, PhD	Eli Lily	Pathway from basic research to drug development
Robert Ring, PhD	Pfizer	Challenges facing autism drug discovery: Industry perspective
Paul Chapman, PhD	Takeda	Discussion leader

Single gene disorders associated with ASD – What can drug discovery?

Mustafa Sahin,MD, PhD	Harvard	Tuberous sclerosis complex
Alcino Silva, PhD	UCLA	mTOR signaling in autism and other psychiatric disorders
Mark Bear, PhD	MIT	Insights from fragile X and related single gene disorders
Huda Zoghbi, MD	Baylor	Rett syndrome and MECP2 Duplication Disorder: Relevance to ASD
Randy Carpenter, MD	Seaside	Discussion leader

Day 1: Topics and Speakers (con't)

ASD genetics and signaling pathways: What are the promising target pathways?

Joe Buxbaum, PhD	Mt. Sinai	Genetics of ASD: Insights into target pathways
Dan Geschwind, MD,PhD	UCLA	Cntnap2 gene knockout as a potential mouse model for ASD
Tom Sudhof, MD	Stanford	Neurexins and Neuroligins – From Synapses to Autism
Randy Blakely, PhD	Vanderbilt	Modeling the Serotonin Driven Traits of Autism in Transgenic Mice
Luis Parada, PhD	U Texas	PTEN pathway
Rene Anand, PhD	Ohio St.	Nicotinic Receptors: Biomarkers and Therapeutic Targets for ASD
Will Spooren, PhD	Roche	Discussion leader

Day 2: Topics and Speakers

Gastrointestinal, immune and metabolic abnormalities, seizures, hormonal systems

Pat Levitt, MD, PhD	USC	Gastrointestinal Conditions and ASD Heterogeneity
Judy Van de Water, PhD	UC Davis	Role of Immune abnormalities
Bob Naviaux, MD, PhD	UC San Diego	Mitochondrial Dysfunction in Children with ASD
Richard Hass, MD	UC San Diego	Autism and Seizures
Evdokia Anagnostou, MD	U Toronto	Oxytocin pathway

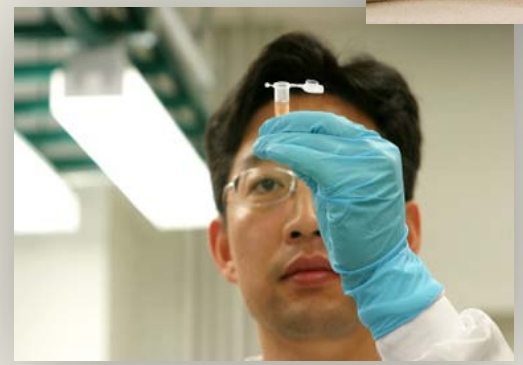
Strategies for target validation: Challenges and promising directions

Jackie Crawley, PhD	NIMH	Behavioral phenotyping in genetic mouse models of ASD
Eric Klann, Ph.D.	NYU	Translational control: Molecules, synapses, and behavior
David Amaral, PhD	UC Davis	Primate models of ASD
Steve Warren, PhD	Emory	Fragile X: Molecular mechanisms and therapeutic implications
Ricardo Dolmetsch, PhD	Stanford	Using iPSCs to study the neurobiology of ASD
Thomas Insel, MD	NIMH	Synthesis and priority needs for accelerating drug discovery

Addressing the gaps and roadblocks

What is needed?

- Promising drug targets related to core and associated features
- Validated animal models
- High throughput drug screening platforms
- Large clinical registries/tissue banks with both phenotypic data and biosamples (DNA, iPSCs, brain tissue, etc.)
- Clinical trial networks
- Biomarkers predictive of treatment response
- Sensitive clinical trial endpoints and surrogate endpoints
- Innovative clinical trial designs (enrichment, stratification, adaptive designs)



AUTISM SPEAKS
It's time to listen.