
**NJ Governor's Council for Medical Research and Treatment of Autism**

# *Proceedings:*

# *Translational Research in Autism*

**The Conference Center at Montclair State University**

**Montclair, New Jersey**

**April 9, 2014**

**PO Box 360, Trenton, NJ 08625-0360**
Telephone 609-633-8740 Fax 609-943-4213 www.nj.gov/health/autism/

# *Purpose for the Meeting*

The goal of all of the initiatives of The Governor's Council for Medical Research and Treatment of Autism (Council) is focused on helping Autism Spectrum Disorder (ASD) affected individuals and their families, and the healthcare systems serving them, to address their needs. Research in the basic, clinical and translational sciences serve as the foundation and first step in discovering best practices in the early identification and treatment of ASD.

The intent of the Council is to raise the level of awareness among stakeholders of the impact of New Jersey autism research while positioning grantees to compete for federal funding. The aim of this conference was to promote new cross-disciplinary collaborations with researchers and clinicians who may be interested in conducting clinical translational research, leading to improvements in interventions that address the physical and behavioral health needs of children, adolescents and adults with ASD. The conference was an opportunity for participants to gain a greater understanding of translational research from national and local experts and to share information and set the stage for possible future collaborations.

Many researchers who attended the first Council sponsored conference in March 2012 also attended this conference. The Proceedings from the 2012 conference are available at http://www.state.nj.us/health/autism/report.shtml. You may find these Proceedings and those from the March 2012 conference helpful as you consider opportunities for autism research.

*The Planning Committee*

- *Madeleine Goldfarb, MA, Chairperson*
- *Liz Bell, B.S.*
- *Karen Hood-Kasim, MPH*
- *Michael Lewis, Ph.D.*
- *Gerald Costa Ph.D.*
- *Martin T. Zanna, M.D., MPH*
- *Linda N. Boclair, M.Ed., MBA*

# Table of Contents


**Autism Spectrum Disorder (ASD) Definition** ..... 4

**The Governor's Council Background** ..... 4

**Council Membership** ..... 6

**Scientific Meeting Agenda** ..... 7

- **Opening Remarks** ..... 8
- **Welcoming Remarks** ..... 10
- **Summary of Presentations** ..... 13
- **NJDOH Remarks** ..... 19
- **Interdisciplinary Group Summations** ..... 20

  **Group 1:** The New DSM Classification System for ASD: What Does it Mean for the Clinician and the Researcher?

  **Group 2:** Early Identification of ASD - Animal Models and Human Behavior: What are the Latest Advances in Genetics and Behavioral Research Which May Aid Us in Identifying Early ASD?

  **Group 3:** New Methods of Intervention: The Role of New Technology for the Delivery of Services

**Council Funding Initiatives** ..... 26

- **New Jersey Autism Center of Excellence (NJACE)** ..... 26
- **NJACE Coordinating Center** ..... 26
- **Clinical Research Program Sites** ..... 26
- **Clinical Research Pilot Projects** ..... 27

**Contact Information** ..... 29

**Appendices:**

**Conference Participants Including Research Interests** ..... 30

**Conference Evaluation** ..... 39

## Autism Spectrum Disorder (ASD)

Autism spectrum disorder (ASD) is a developmental disability with early childhood onset. A diagnosis of ASD includes several conditions as defined by the DSM-5 (The American Psychiatric Association's Diagnostic and Statistical Manual, Fifth Edition): autistic disorder, pervasive developmental disorder not otherwise specified (PDD-NOS), and Asperger syndrome. These conditions are all called autism spectrum disorder. The prevalence of ASD may be increasing, and ASD is more common than previously thought. Autism spectrum disorder, for which there is presently no cure and only limited treatments, generally have lifelong effects. The Centers for Disease Control and Prevention (CDC) estimates that an average of 1 in 68 children in the United States has an ASD. As part of the same CDC study, the prevalence rate for the New Jersey sites was established at 1 in 45 children (MMWR, March 28, 2014 / 63(SS02);1-21).

## Governor's Council for Medical Research and Treatment of Autism

The Governor's Council for Medical Research and Treatment of Autism (Council) was created by State appropriation in 1999 and has been issuing research, clinical and educational enhancement grants since 2000. The Council is in the New Jersey State Department of Health (NJDOH).

In 2012 the Council established the New Jersey Autism Center of Excellence (NJ ACE) whose mission is to research, apply and advance best practices in the understanding, prevention, evaluation and treatment of autism spectrum disorder, enhancing the lives of individuals across their lifespans. To this end, the Council awards grants and contracts to public and private nonprofit entities. As per P.L. 2007, c.174, monies from $1 surcharges on fines and penalties from traffic violations are deposited by the State Treasurer into the Autism Medical Research and Treatment Fund to sponsor the Council to fund autism research and treatment in the State of New Jersey.

As part of its enabling legislation, the Executive Director of the Council has the responsibility for establishing a Scientific Advisory Committee (SAC). The SAC includes three biomedical research scientists with demonstrated achievements in biomedical research relating to autism and two medical clinicians whose practice is primarily devoted to the treatment of individuals with autism. The SAC identifies and makes recommendations, through the Executive Director, to the Council regarding grants. These recommendations by the SAC are intended as guidance to the

deliberations of the Council, which is responsible for decisions on funding of programs and grants.

Please refer to the Council's website (www.nj.gov/health/autism) for additional information including the Council's Meeting Calendar. The meetings are open to the public.

## Council Membership

**Elizabeth K. Bell, BS**, Autism Organization Representative; Volunteer and Independent Contractor with Autism Speaks.

***Caroline Eggerding, M.D.,** Healthcare Organization Representative; Division Head, Department of Pediatric Neurology and Development, Cooper Children's Regional Hospital.

**Susan P. Evans, Ed.D.,** Commissioner of Health Appointee, and the Education Program Specialist for Early Intervention program in the NJDOH.

**B. Madeleine Goldfarb, MA,** Autism Organization Representative; Founder/Director of the Noah's Ark Children's Association.

**Ketan Kansagra, M.D., FAAP,** Academic Institution Representative; Division of Neonatal Medicine, Children's Hospital of New Jersey at Newark Beth Israel Medical Center.

**Gary Weitzen, BA,** Autism Organization Representative; Executive Director of POAC (Parents of Autistic Children).

* Denotes Council Chair

## Council Staff

**Martin T. Zanna, M.D., MPH**
Acting Executive Director, Governor's Council for Medical Research and Treatment of Autism
**Linda N. Boclair, M.Ed., MBA**
Executive Assistant, Governor's Council for Medical Research and Treatment of Autism
**Mary Ray**
Contract Administrator II, Governor's Council for Medical Research and Treatment of Autism

# *Translational Research in Autism*

**Conference Agenda**
**April 9, 2014**

**8:00 am** Registration and continental breakfast

**9:00 am** Opening remarks.....Caroline Eggerding M.D.
Chairperson, Governor's Council for Medical Research and Treatment of Autism

Welcome.....B.Madeleine Goldfarb, MA
Member, Governor's Council for Medical Research and Treatment of Autism
Chairperson, Planning Committee

**9:15 am** *Pathways toward Translational Research Programs for Autism Spectrum Disorder*
Helen Tager-Flusberg, Ph.D. Professor, Department of Psychological and Brain Sciences, Boston University; Director, Center for Autism Research Excellence.

**10:00 am** *Translational Research: A National Perspective* ..... Alycia Halladay Ph.D.
Senior Director of Research
Autism Speaks, NYC

**10:45 am** *NJDOH Remarks*.....Cathleen Bennett, Director of Policy
New Jersey Department of Health

**11:45 am** *Interdisciplinary Discussion Group*.....Michael Lewis, Ph.D.
University Distinguished Professor of Pediatrics and Psychiatry; Director of the Rutgers Robert Wood Johnson Medical School Autism Center; Director of the Institute for the Study of Child Development, Rutgers-RWJMS

**Group Leaders:** Group 1- Audrey Mars, M.D., Medical Director, Hunterdon Medical Center Developmental Pediatric Associates; Clinical Associate Professor of Pediatrics, Voluntary staff, Rutgers-RWJMS, Group 2- James Millonig, Ph.D., Associate Professor, Rutgers-RWJMS Neuroscience & Cell Biology, Group 3- Thomas McCool, Ed.D., President Emeritus, Eden Autism Services.

**1:00 pm** Lunch

**2:00 pm** Group Discussions, cont. and Reports

**3:45 pm** Closing remarks

**4:00 pm** Adjournment

## Opening Remarks

Caroline Eggerding, M.D., Chairperson, Governor's Council for Medical Research and Treatment of Autism

Welcome on behalf of the members of the NJ Governor's Council for Medical Research and Treatment of Autism (Council). Personally this is an extraordinary opportunity for me to hear from all.

The Council was formed in 1999 by legislative statute. Initially it was situated in the University of Medicine and Dentistry of New Jersey. In 2007 the Council was moved to the Department of Health. Membership increased to 14 representatives from academic institutions, healthcare and autism advocacy organizations. In addition, there is representation from the public and includes a person affected by autism. There are also appointees from the Senate President, Assembly Speaker and Commissioner of Health.

The Council's mission is to create an Autism Center of Excellence in the State to support basic and applied research, diagnosis and treatment initiatives. The Council has the responsibility to award grants and contracts to public and private nonprofit entities. The initiatives are funded by a $1 surcharges on fines and penalties from traffic violations, resulting in a $4 million budget dedicated annually to autism.

Autism was first described in 1943. Since then there have been over 34,000 publications in the scientific literature that reference autism, half of these occurring since 2008. With all of this we are still not entirely in agreement about how we diagnose autism, what causes autism and how it is best treated. The CDC just reported the most recent numbers which I am sure you are fully aware of. Autism affects 1:68 people in the United States. Actually it affects us all. It affects us as individuals with autism, as family and friends and as scientists and citizens.
There is urgency in our work. There is great urgency in our working together. If it was easy, we would have resolved this with our 34,000 studies. It is hard. We still have much to do. This is the time. I can't wait to see the answers. And I don't want to be freeze dried and reconstituted in 100 years to hear the rest of the story. I am selfish. I want to know.

*The CDC just reported the most recent numbers which I am sure you are fully aware of. Autism affects 1:68 people in the United States. Actually it affects us all. It affects us as individuals with autism, as family and friends and as scientists and citizens.*

Neil deGrasse Tyson has said so many wonderful things about the role of science in our lives but I think this one fits our shared work:

"For me, I am driven by two main philosophies: know more today about the world than I knew yesterday and lesson the suffering of others. You be surprised how far that gets you."

It is now my privilege to introduce Madeleine Goldfarb.

Ms. Goldfarb is the Founder and Executive Director of The Noah's Ark Institute. She has long been an advocate for children and families with autism and a dedicated and active member of the NJ Governor's Council for Medical Research and Treatment of Autism. She is a member of The Centers for Disease Control's (CDC) Learn Signs - Act Early State Teams and of the Advisory Board of the Daniel Jordan Fiddle Foundation as well as working with FEMA (Federal Emergency Management Agency) in educating first responders. Most recently Ms. Goldfarb spearheaded efforts to acknowledge the successes of New Jersey autism researchers, with her efforts culminating in today's meeting. And now Madeleine will share her thoughts and personal experiences in working with the autism community including families, researchers and providers.

## Welcome

B. Madeleine Goldfarb, MA
Member, Governor's Council for Medical Research and Treatment of Autism
Executive Director, Noah's Ark Institute

It is my pleasure to welcome you all to the second scientific gathering of The Governor's Council for Medical Research and Treatment of Autism. It is so wonderful to see so many new faces. It is wonderful to see the old faces as well. It is wonderful to see so many of the old faces looking so new. You all look marvelous! That also means we have held your interest and you have continued your work to shine light into the dark regions of our knowledge base on autism.

I want to thank the Council for once again giving me the opportunity to bring you together and foment dialog between you and your colleagues. I always like to tell you how the work of the Council and the Council funded projects receive their funding. In short....bad driving. Yes, every time a moving violation is written in the State of New Jersey the Council receives one dollar. So, if you have been issued a citation, I thank you, the Council thanks you, and your colleagues thank you. Plus, now you don't have to feel so bad about it. Also of note, is that year after year New Jersey drivers do not seem to be able to improve their driving skills, as the funding for the Council has been remarkably consistent over time. New Jersey is unique in the opportunity this funding presents to our State and our residents as well as giving New Jersey researchers the opportunity to be thought leaders at the forefront of autism research, not only here in New Jersey, or the country but around the world.

*New Jersey is unique in the opportunity this funding presents to our State and our residents as well as giving New Jersey researchers the opportunity to be thought leaders at the forefront of autism research, not only here in New Jersey, or the country but around the world.*

You are leaders in your profession and in the work that the Council has been able to inspire you to undertake. If you did not start out on the path of autism and we have brought you to this path we are the richer to have your energies here asking questions and finding answers.

Raising a child with autism is not for the faint of heart. It takes bravery, tenacity, guts and a dose of zeal. If you want something done, give it to a parent of a child with autism. Never underestimate the power of autism as we will move the world and change it for the better in our wake. We will create a council which funds autism research, we will read every word written in your proposals and in your research, we will be acquainted with every detail of every project and we will advocate for you with unyielding perseverance. We are your staunchest supporters. Never doubt that we need each other.

We expect so much of you, but what can I give in return. I can give only a perspective. I want to tell you about autism. You cannot understand autism unless you live with autism. You cannot walk in our shoes. We are most grateful for your walking beside us, but you cannot know the true urgency of your work until you glimpse our world, our reality, our days, our nights, our futures and the future of every person with autism.

I want to share a story with you...

*The days of play and laughter were replaced by movements and jerks from an unknown anguish.*

Once upon a time...There was a little boy, full of love and life and joy. He was the most beautiful boy the world had ever seen. One day before his second birthday the beautiful boy, of such love and life and joy, for reasons his parents could not explain, slowly turned inward, listening to another calling. This inner voice was seemingly not of this world, yet, it was so compelling to the beautiful boy, full of love and life and joy, that he listened harder and harder every day until he barely came back into the world of his parents. The days of play and laughter were replaced by movements and jerks from an unknown anguish. Screams and cries and fear bubbled up within his being from the depths of nowhere. The night which had been a peaceful refuge was now full of teeming storms in the beautiful boy's head, and he did not sleep, and his parents did not sleep and the turmoil was full and great as the months and months went by.

Time goes slowly in the present and so quickly in reflection. The beautiful boy grew, and as in all families, on the mantel of the hearth, there were pictures. There stood one buried in the back, hidden behind all the others, of the beautiful boy, full of love and life and joy. The mother picked up the picture and gazed at the beautiful boy, full of love and life and joy, frozen forever in time, with tears streaming freely down her checks. She mourned the loss of the beautiful boy, full of love and life and joy and of the path that was diverted when the beautiful boy was called inward. As she looked up to put the picture back behind all the others she was filled again with tears, only these were the tears of love and hope and gratitude of the beautiful boy who grew into a tall handsome young man who overcame so many herculean obstacles and thrived and progressed more than anyone thought was his right and he proved what is possible and she knew that her tall handsome young man was a light and a blessing in this world.

*...in New Jersey where...one in 45 children and one in every 28 boys full of love and life and joy will be given this diagnosis...*

One family...one story in New Jersey where by the time they are eight years old every one in 45 children and one in every 28 boys full of love and life and joy will be given this diagnosis and start one more family on a journey fraught with fear, anxiety, misunderstanding, and

uncertainty. If they are truly lucky there will be joy at the miraculous steps forward. There will always be love for their child who may just beat the odds. In the future the miraculous progress will not be based on luck. That progress will be based on your science.

*You (researchers) give us the most precious gift...The gift of knowledge, perspective and yes the audacity to hope.*

You give us the most precious gift...the gift of knowledge, perspective and yes the audacity to hope. We hope not only for our own children but for the unending tide of children being diagnosed with autism in record numbers without seeming end or even ebb. We hope for the future of the massive wave of young adults aging out of their school age entitlements to an uncertain future.

They all are in your hands. Take care of them as we do, by never giving up. Match the countless sleepless nights of families in the throes of autism by your relentless pursuit of knowledge. Match our unending struggle with therapy and appointments, educational challenges, and aging difficulties with your indomitable inquiry. Match what we do every day with your unwavering zeal and tenacity to give us the answers so we can finally rest.

Research is a sip of water that leaves you parched. The drink is wet but your thirst is never quenched.

Drink on!!! Drink on!!!

In closing I wish you a productive meeting with insightful discussion and the blossom of collaboration in your future endeavors.

Thank you.

**Pathways toward Translational Research Programs for Autism Spectrum Disorder**

Helen Tager-Flusberg, Ph.D. Professor
Department of Psychological and Brain Sciences, Boston University
Director, Center for Autism Research Excellence

For several decades after autism was first described by Kanner, research was severely limited by lack of awareness, interest, funding, theoretical frameworks and tools for investigating etiology, pathophysiology or mechanisms. More recently, the exponential rise in prevalence rates, which brought widespread concomitant concerns about this neurodevelopmental disorder, funding levels for research grew and publications focusing on all aspects of ASD appeared. At this stage, given the current rates of 1 in 68 children receiving a diagnosis of ASD, it is now imperative for research to move beyond asking what, why and when, and begin addressing the most significant research issue for families: How can we make a difference in the lives of children with ASD through the development of new treatments?

The goals for research on treatment include the creation of safe developmentally sensitive individualized (personalized) treatments that target the biological and behavioral phenotypes of each child and adult producing significant changes in behavior with few side effects. These goals can be met through translational research programs, which can take several different pathways. In this presentation I highlighted three pathways:

1. Behavioral treatment models that are grounded in basic developmental science
2. Pharmacological treatments that are grounded in molecular biology
3. Neurological-based treatments that are grounded in knowledge about neurocognitive architecture.

Behavioral Treatments

Dr. O. Ivar Lovaas first demonstrated the efficacy of classic applied behavioral analysis (ABA) approaches (discrete trial training) to treating ASD, which produced significant outcomes in preschoolers with ASD. In general, ABA is a methodology for producing behavioral changes, and has repeatedly been shown to be an effective set of techniques for improving cognitive and language skills in children with ASD, however the content of ABA programs is not well grounded in what is now known from the developmental sciences. More recently, newer behavioral treatments have translated what is known about the core developmental impairments in ASD, many of which are recognized to be critical precursor skills for language and communication including imitation, joint attention, play and gestural developments. Developmental science has demonstrated that learning in infants and young children is an active process that takes place in rich social-affective contexts and new treatment approaches have been created to bring these basic insights into ASD treatment paradigms.

One excellent example of a comprehensive specialized treatment model was tested in a randomized controlled trial (RCT) by Dawson and colleagues. Forty-eight toddlers with ASD, whose average age was 24 months, were randomized to either the comprehensive treatment model, grounded in developmental science (Early Start Denver Model - ESDM) or to a community control treatment for two years. ESDM targets multiple core behaviors delivered in a structured affectively-rich context. Behavioral changes in the infants were evaluated after 12 and 24 months, and brain changes were evaluated after 24 months of treatment. The toddlers in the ESDM group gained on average 20 IQ points, and their adaptive skills on the Vineland did not decline over the two years of treatment. These changes were significantly greater than the effects seen in the control group. Moreover, ESDM was also shown to normalize brain activity in response to faces and objects in preschoolers with ASD.

There are other examples of novel behavioral treatment approaches that are based on advances in research in developmental science. Kasari and her colleagues implemented an intervention to train parents to increase joint engagement with their ASD children, which they tested in a RCT involving 20 sessions each lasting 30 minutes. The intervention led to increases in joint attention, in comparison to a waiting list control group that continued to grow even after the intervention was completed. Taken together, these and other recent behavioral intervention models demonstrate how effectively basic research in developmental sciences have been translated into novel treatment approaches.

Pharmacological Treatments

In medicine, the "bench to bedside" model has become the most widely known translational research model. This approach follows the path from gene discovery to the creation of a mouse model of the mutated/absent gene(s), which leads to deep understanding of the underlying disease pathophysiology that could translate into novel drug development that can be tested first in animal models and then in human clinical trials.

One neurodevelopmental disorder that has followed this pathway is fragile X syndrome. This disorder is directly relevant to ASD because about 40% of individuals with fragile X syndrome meet diagnostic criteria for ASD, and even in those individuals without co-morbid ASD, the core phenotype includes social and behavioral symptoms that parallel the defining features of ASD. Fragile X syndrome is caused by mutations (excessive number of a triplet repeat pattern on the FMR1 gene located on the X chromosome). The story of how this gene discovery was taken on the translational research journey was led by the neuroscientist Mark Bear. There are now several clinical trials involving drugs selected for targeting the synaptic pathophysiology that have been completed or are still underway. To date, the results have been disappointing and there is still no FDA approved drug treatment for fragile X syndrome or

ASD that reduces core symptoms of the disorders. There are several challenges that need to be overcome for pharmacologically based translational research. These include identifying appropriate and sensitive outcome measures, avoiding significant placebo effects, figuring out how early such treatments could begin, whether people would be required to stay on the pharmacological treatment for their entire lives and associated ethical issues related to such treatment.

Neurologically Based Treatments

A third pathway to translational intervention research on ASD focuses on the neuro-cognitive architecture that underlies ASD-related deficits. This is the approach taken in our current NIH-funded Autism Center of Excellence (ACE), which addresses the question of why up to one-quarter of all children with ASD fail to acquire spoken language. Our conceptual framework builds on what is known about the complex neural systems that are involved in processing language from auditory input to spoken output. In our ACE we are testing several hypotheses about the specific mechanisms that may underlie the speech and language deficits in minimally verbal children with ASD in the context of a novel intervention that itself is grounded in what is known about the close links between speech, motor and music cognition.

The intervention is called 'auditory-motor mapping training' or AMMT in which associations between sounds and articulatory actions are training by incorporating both song and manual motor actions into the training, delivered in a structured (ABA) socially engaging context. Two hypotheses about mechanisms that might be impaired in minimally verbal children will be tested, each of which is grounded in basic computational neuroscience. One focuses on the speech output system (using fMRI and DTI), the other on the processing of auditory input (using electrophysiology). The neural assessments are administered pre- and post-intervention to test whether AMMT leads to changes in the neural networks that underlie the speech or auditory systems being investigated.

To summarize: scientific knowledge about ASD has advanced exponentially over the past decade. We are now ready to integrate that knowledge and identify novel treatment approaches and paradigms that translate across levels from biology to behavior. Much of the increased support for research has been driven by families that are directly affected by ASD who have prompted both private foundations and public (federal, state) research support. We now owe these families the opportunity to participate now in the next generation of translational research programs. Please refer to the following link for Dr.Tager-Flusberg's slide presentation: HPathways Toward Translational Research Programs for ASD.pdf

**Translational Research: A National Perspective**

Alycia Halladay Ph.D.
Senior Director of Research at Autism Speaks, NYC

Translational medicine is defined as a discipline within biomedical and public health research that aims to improve the health of individuals and the community by "translating" findings into diagnostic tools, medicines, procedures, policies and education. This can mean different things to different people, disciplines and organizations. For a funding agency that supports different type of research, it means: how can we get findings in a clinic or laboratory environment to people around the globe that could benefit from it? In other words, bringing findings from bench to bedside.

There is something known as the "valley of death" in translational research. There are research organizations and foundations that typically fund discovery research. This includes identification of genes, methodologies, prevalence estimates, risk factors, etc. There are also billions of dollars of investment in capitalizing the findings into commercial products – or putting them into bedside. The middle phase, called "development" is where many products or investments fail. To bring a discovery to the next phase requires a large amount of oversight, financial investment, and expertise. Commercial entities are sometimes reluctant to move findings into this development phase, and in many cases, there is concern that adequate discovery has been accomplished. So how can a not for profit organization "de risk" this phase and help with the development of discoveries into products?

In order to address this challenge, Autism Speaks is engaged in four major activities, the first three of which were described in the presentation: 1) supporting commercial endeavors in the for-profit space, 2) funding junior researchers through post-doctoral fellowships who focus on translational research project and 3) developing infrastructure for clinical trials research to test or develop new compounds or medical practices in a large scale.

The first example is a LLC founded by Autism Speaks called DELSIA (Delivering Solutions in Autism). The goal is to support commercial entities through the development phase for products and innovations specific to people with ASD and their families. An LLC was established to protect the parent organization, Autism Speaks, from risks associated with this type of investment, however, potential profits could be directed back to the organization for further research opportunities. This solution may not be suitable for all organizations. To complement translational research in the academic setting, a translational post-doctoral fellowship mechanism was established. This serves 2 purposes: 1) provides support for new researchers and 2) focuses research to four major translational activities. These are Biomarker Discovery,

outcome measure development, preclinical target validation and experimental therapeutics. Examples of projects in each activity were presented.

The final way in which Autism Speaks is supporting translational medicine is the development of clinical trial infrastructure for autism researchers. This includes the Autism Treatment Network (ATN). This is a network of 17 hospitals across North America which contributes data from families to a common registry. Because it is a hospital based model, the network focuses on the medical comorbidities that accompany autism. They also utilize a standardized protocol for assessment of patients. A modest amount of funding is provided by Autism Speaks for the coordination of care for patients, additional monies go to the database. Research funding is provided by HRSA (Health Resources and Services Administration) who use the ATN platform to study research questions around GI dysfunction, sleep and diet in ASD. To provide resources to the community, the ATN has developed a set of toolkits that are disseminated to the community. These toolkits receive several thousand downloads each year, and are focused on the medical needs of children with ASD.

Because they are a live, captive audience already deeply engaged in research and affiliated with well-respected clinicians across the nation the ATN sites are a very attractive source of patients for clinical trials during the development phase of translational medicine.

Another way in which Autism Speaks supports translational research via a similar mechanism, but not mentioned in the talk can be seen in the investments in early identification and intervention. In 2003, Autism Speaks and the NICHD partnered to form the Baby Siblings Research Consortium (BSRC). This is a network of now 25 researchers at 23 research sites across the world. These investigators are funded to collect data through other mechanisms, and for a modest investment (a steal, really), Autism Speaks funds a database which houses all of the data for collaborative use. Out of this investment we now know that 1/5 siblings of someone with ASD develops an ASD themselves, and another 1/3 has developmental issues that is not diagnosed as autism but warrants attention. Currently, the database houses information on 4000 high risk siblings and 1000 low risk siblings.

All of the research sites in the BSRC provides or facilitates early intervention services for children as young as 1 year and up to 3 years of age. Little has been understood about which of these interventions are effective, how they are best delivered, and how outcome should be measured. In 2007 Autism Speaks offered an RFA to evaluate the efficacy in randomized clinical trials of different early intervention protocols, both among the BSRC researchers and other interested study sites. As there were common elements across the studies, including the common research design of parent-mediated interventions, the funded researchers established the Toddler Treatment Network (TTN). By gathering and pooling their datasets, they have led

to comparative efficacy studies. The network now is in the development stage by disseminating evidence based intervention practices through focused interactions with community based providers. The network has also expanded beyond the original Autism Speaks funded projects to encompass a diverse set of both interventions and study populations.

With both the ATN and TTN models, Autism Speaks has provided financial support (as well as administrative support in house) to facilitate infrastructure needed for generating data. The investment can be modest or intensive, depending on the model, needed costs, and leveraged support. However, they serve multiple purposes for both discovery and development research. Please refer to the following link for Dr.Halladay's slide presentation: **Translational Research in Autism. A national perspective.ppt**

**Autism Research and Services - New Jersey Department of Health**

Cathleen Bennett, Director of Policy and Strategic Planning

Good morning. On behalf of the New Jersey Department of Health (NJDOH) I welcome you and thank you for attending today's conference. And a special "thank you" to the Council's Conference Committee for their work in planning this event and providing an opportunity for you to gain a greater understanding of translational research, share information and set the stage for possible future collaborations.

Over $8M has been awarded for autism research by the Council and the NJDOH since 2012, including funding to establish the New Jersey Autism Center of Excellence (NJACE) with Montclair State University serving as the Coordinating Center for the NJACE. We anticipate up to an additional $4M will be awarded for clinical translational research projects within the next couple of months. During the past year, the state has worked closely with the NJACE Coordinating Center at Montclair to build out the capabilities of the NJACE to include translational research to help researchers translate what they have learned in their controlled lab studies to other settings, such as schools or provider offices. The NJACE's translational research capability is the critical next step towards real world application of research discoveries.

*The NJACE's translational research capability is the critical next step towards real world application of research discoveries.*

For autism services, New Jersey has one of the best systems in the nation for identifying, diagnosing and documenting children with autism spectrum disorder. Under the leadership of Governor Christie, the state has committed $135M for the Department's Early Intervention System, which provides early identification and referral, service coordination, evaluation and assessment for children from birth to age three with developmental delays and disabilities. New Jersey is one of only four states with an Autism Registry that requires reporting by neurologists, pediatricians, nurses and other autism providers so children can be referred for resources and services. Approximately, 12,400 individuals through age 21 are registered. Each child who is registered is referred to the Special Child Health Services Case Management Unit within the county of residence where the case managers assist them in accessing services, including referrals to the Early Intervention Program.

*For autism services, New Jersey has one of the best systems in the nation for identifying, diagnosing and documenting children with autism spectrum disorder.*

Thank you for your attention. I hope to see you at the 2015 autism conference.

## Interdisciplinary Discussion Groups Summation

Interdisciplinary Discussion Group..... .Michael Lewis, Ph.D.
University Distinguished Professor of Pediatrics and Psychiatry; Director of the Rutgers Robert Wood Johnson Medical School Autism Center; Director of the Institute for the Study of Child Development, Rutgers-RWJMS

**Group Leaders:** Group 1- **Audrey Mars, M.D.**, Medical Director, Hunterdon Medical Center Developmental Pediatric Associates; Clinical Associate Professor of Pediatrics, Voluntary staff, Rutgers-RWJMS, Group 2- **James Millonig, Ph.D.**, Associate Professor, Rutgers-RWJMS Neuroscience & Cell Biology, Group 3- **Thomas McCool, Ed.D.**, President of TMC Consulting LLC, Past President/CEO, Eden Autism Services

**Group 1:** The New DSM Classification System for ASD: What Does it Mean for the Clinician and the Researcher?

**Group 2:** Early Identification of ASD - Animal Models and Human Behavior: What are the Latest Advances in Genetics and Behavioral Research Which May Aid Us in Identifying Early ASD?

**Group 3:** New Methods of Intervention: The Role of New Technology for the Delivery of Services.

**Introduction by Michael Lewis, Ph.D.**

As mentioned earlier the goal of this conference is to promote new cross-disciplinary collaborations with researchers and clinicians who may be interested in conducting clinical translational research. Each group had been assigned questions to guide the discussions. These are the "big questions" currently being asked in autism. Our intent is for you to achieve a better understanding of the topics and explore opportunities toward working together.

**Group 1 Question:** The New DSM Classification System for ASD: What Does it Mean for the Clinician and the Researcher?
**Reporting:** Audrey Mars, M.D., Medical Director, Hunterdon Medical Center Developmental Pediatric Associates; Clinical Associate Professor of Pediatrics, Voluntary staff, Rutgers- RWJMS.
**Members**: Gerard Costa, Ph.D., Agnes Cushing-Ruby, Emanuel DiCicco-Bloom, M.D., Karen Hood-Kasim, MPH, Sandra Howell, Ph.D., Andrea Lubin, Alexandria McEntee, Mark Mintz, M.D., Carolyn M. Salafia MS, M.D., Nancy Scotto-Rosato, Ph.D., Venkat Venkataraman Ph.D., Harvey R. Weiss, Ph.D. and Barbie Zimmerman-Bier, M.D.

Members of the group included both clinicians and basic science researchers with varying familiarity of DSM-5 criteria. The fifth edition of the DSM-5 criteria was published in May 2013. The DSM IV-TR criteria included diagnostic subcategories (PDD-NOS, Asperger's Disorder and Autistic Disorder). The DSM-5 has a single broad category of autism spectrum disorder (ASD).

The DSM IV-TR had 3 symptoms domains (social, communication and atypical behaviors). The DSM-5 has 2 (social/ communication deficits have been combined into one domain; the other domain is restricted repetitive behaviors (RRB) which does include sensory symptoms. In order to confirm a diagnosis the new criteria requires all symptom clusters in the social/communication domain be present and two of four symptoms in the RRB cluster to be present.

It was emphasized in the DSM-5 criteria that individuals previously confirmed to have a diagnosis of AD, PDD-NOS or Asperger's Disorder will continue to have diagnosis of ASD. Individuals previously diagnosed do not have to get a new diagnostic evaluation due to the new diagnostic classification unless it is clinically indicated. Despite this, there are reports of clinicians and schools that have requested reevaluations.

Workshop members expressed concerns regarding the diagnostic criteria resulting in all individuals with autism being classified in one category. Presently, there are broad classifications and the focus of DSM-5 criteria is on the specific behaviors or deficits. The clinicians emphasize that diagnosis is to be based on whether behaviors/deficits interfere with function.

The clinicians present expressed concerns that children are being forced into a classification category in order to obtain educational services and to obtain healthcare reimbursement. A particular area of concern is that the new criteria may present a barrier to identify young children under the age of three so they can receive appropriate early intervention services. In the DSM-5, language ability is no longer part of the criteria. Language delay makes it challenging to evaluate the child's social communication and peer engagement. In the young child there is a need to focus on play skills appropriate to developmental level.

The clinicians present were concerned that, for individuals who do not meet the full criteria based on the DSM-5 criteria for ASD, their educational and behavioral services be based on need and not diagnosis. The child's level of hyperactivity is another factor impacting behavior and social reciprocity.

Among the researchers present, discussion was focused on the DSM-5 criteria not being sufficient for identification of subject recruitment. Standardized tools will continue to be utilized (ADOS-2; ADI-R). Other issues discussed were about the impact of a broader diagnostic group. When individuals are categorized together in a broad group, a larger sample size is needed. In genetic studies there is a need to look at the underlying genotypes in order to understand the biology than to move backwards to the phenotypes (behavioral characteristics).

Another concern expressed was that the severity score was not being implemented in a standardized way. This is especially important in younger age children since it is hard to distinguish support needed that is specific to ASD versus a young child's developmental needs

The presence of a severity code in ADOS-2 was highlighted as a positive way to track individuals over time. However the ADOS-2 is looking at behavior only at the time of testing. The DSM-5 Classification is dependent on both current behaviors and those that have occurred in the past. The ADI-R interview continues to be labor intensive and limited usage in routine clinical practice.

Additional discussion focused on the NJ autism registry and the need to include the DSM-5 criteria for children to be registered. Concerns were raised particularly with the young children since initially they may be identified to be at risk. However, Individuals need a confirmed diagnosis to be registered.

The IACC has recently highlighted areas for potential research regarding changes to the DSM-5 criteria. The National Institutes of Health has invited input from the public, including families, clinicians and researchers on research that can explore the effects of the DSM-5 criteria for individuals with ASD.

**Group 2 Question:** Early Identification of ASD - Animal Models and Human Behavior: What are the Latest Advances in Genetics and Behavioral Research Which May Aid Us in Identifying Early ASD?
**Reporting:** James Millonig, Ph.D., Associate Professor, Rutgers-RWJMS Neuroscience & Cell Biology
**Members**: Linda Brzustowicz, M.D., Gail Burack, Ph.D., Gabriella D'Arcangelo Ph.D., Bonnie L. Firestein, Ph.D., Judy Flax, Ph.D., Jill Harris, Ph.D., Lisa Huguenin, Ph.D., Yvette Janvier, M.D., Harumi Jyonouchi, M.D., Elizabeth M. Lennon, Ph.D., Eric London M.D. and Nicholas M. Ponzio, Ph.D.

The charge of Group 2 was to discuss methodologies that may assist in early detection of autism. The group agreed that the heterogeneity of the disorder makes this goal difficult. The group then went on to discuss how genetics, mouse models, human stem cells and early behavioral phenotypes may be used to address this charge.

Genetics: Advances in sequencing technology and computational ability now make it possible to sequence the entire genome and identify genetic variants at a reasonably low cost. However we lack the tools to discriminate between silent and functional variants that contribute to autism risk. This inability makes it impossible to use genetics alone to predict autism risk. We agreed that new bioinformatics tools are needed along with molecular analysis to identify functional variants that contribute to risk

Mouse models: Mouse models are typically used in autism studies to determine the function of genes and genetic variants during brain development. The mouse provides one of the few systems that allow the investigation of these questions at an organismal and tissue level. However the high cost and low throughput of the system are considerable drawbacks. The group suggested that functional studies are also needed in lower cost high throughput complementary systems.

iPSCs: Induced pluripotent stem cells (iPSCs) provide a new model for examining the effect of human mutations during neurodevelopment. While the full utility of the system still needs to be determined, iPSCs could provide an opportunity to identify cell biological and molecular pathways associated with autism. These phenotypes could potentially be biomarkers for risk and entry points for treatment development. However high throughput low cost methodologies are needed for iPSC generation if it is going to be used as a system to help identify individuals at risk for autism. In addition these studies are a long way away from human treatments and the group acknowledged that phenotypes in humans change over time so applying the system to treatments may be difficult

Early endophenotypes: Several groups are using early behavioral endophenotypes to identify individuals at risk for autism. Examples include: placental tissue which can reflect the obstetric history and hinting on the developmental potential of the child, (2) early EEG study for imaging brain in the resting versus and (3) infant eye movement tracking. These assays are still under development and it is currently unknown the predictive power of each phenotype given the heterogeneity of the disorder

Finally difficulties on the ground in providing the services make translating any findings into new treatments difficult. The group agreed that this needs to be addressed in the future.

Everyone agreed that the best route forward is a combinatorial approach that includes all the topics discussed above since it would leverage the most information and possibly deal with the heterogeneity of the disorder. However to successfully leverage data from all these resources, recruitment of statisticians and bioinformatists that can handle and mine large datasets is needed.

**Group 3 Question:** New Methods of Intervention: The Role of New Technology for the Delivery of Services.

**Reporting:** Thomas McCool, Ed.D., President of TMC Consulting LLC
Past President/CEO, Eden Autism Services

**Members**: Oana de Vinck-Baroody, D.O., Cecilia Feeley, Laura Henderson, MA, BCBA, Randye Huron, M.D., MaryLou Kerwin, Ph.D., Melanie McGackin, Heather McGowan, Pnina Mintz, Ph.D., Zhiping Pang, Ph.D., Nicole Pelliciari, T. Peter Stein, Ph.D. and Jonathan Sabin

Technology has become an integral tool in many facets of service delivery within the autism services arena, including:

1. Communication
2. Promoting Positive Behavior
3. Demonstrating Social Skills
4. Teaching academics
5. Teaching employment skills
6. Promoting access to employment

In addition, technology plays a major role in the training of teachers, families, and direct services professionals. Courses and testing is available for the following:

1. Board Certified Behavior Analysts – courses from various sources; certification from the Behavior Analyst Certification Board
2. Qualified Autism Services Practitioner and Applied Behavior Analysis Technician – courses from various sources; certification since December 2012 by the Qualified Applied Behavior Analyst Certification Board, and beginning April 2014 by the Behavior Analyst Certification Board.
3. Teachers – Senator Ruiz has introduced a bill (SB1039) in New Jersey to require special training for teachers to whom children with autism are assigned. This legislation provides for the issuance of an autism teacher authorization for teachers who meet the educational requirements.

Video streaming technology – can provide access to children at school or in the home so that behavior programs can be monitored and evaluated. In addition, these video streams can provide access to staff delivering services to ascertain the effectiveness of the service and the personnel delivering the service. CNNH currently utilizes video monitoring in schools and homes.

Individualization – the group discussed a number of Apps and programs in place that provide for individualization of instruction and/or information sharing including TinyTap – an App that lets you make your own App.

First Responders Training – we discussed the implementation of legislation requiring all first responders to take specific autism training. It is questionable that the available training is enough or effective.

Telemedicine – technology currently permits the delivery of telemedicine and is particularly helpful in rural areas where there is limited access to medical professionals. One impediment to expanding these services is the practice of paying less for services delivered in this way. The American Telemedicine Association is supporting the passage of state legislation mandating parity in payment for medical services.

Shared portal – there was general agreement that there is a great deal of information available through technology but it is not organized or easily accessible in terms of specifics.
Resources:

1. Verizon – has recently completed its Powerful Answers contest and has awarded significant capital to small companies working to provide programs for autism. Two of the award winners are:
   - TinyTap – an App that lets you make your own Apps
   - Web Team – a number of teaching Apps developed using the Eden Autism Curriculum to identify teaching activities included in the curriculum. Can be accessed at: www.webteamcorp.com
2. edWeb – a social network for several "communities" one of which is an autism community in which more than 2000 teachers of children with autism in both public and private schools share information and seek assistance with specific classroom issues. Can be accessed at: www.edweb.net
3. MuseAmi – a software development company that began with a focus on music programming, and expanded into using its technology in speech terapy.

Public policy support and funding for technology – requires that there be cost effective and cost efficient outcomes.

## The Council's Funding Initiatives (2012-2017)

**New Jersey Autism Center of Excellence (NJ ACE).** This five year NJ ACE initiative represents the Council's and the State's commitment to advancing the current knowledge pool through clinical and translational research. It is anticipated that the initiative will lead to study outcomes that will have direct clinical impact such as earlier diagnosis and improvements in interventions that address the physical and behavioral health needs of children, adolescents and adults with ASD.

**NJ ACE Coordinating Center.** In June 2012 The Center for Autism and Early Childhood Mental Health at Montclair State University was awarded $1.5 million over 5 years to establish the NJ ACE Coordinating Center. The NJ ACE Coordinating Center provides common management and support functions in order to unify the NJ ACE grantees (Clinical Research Program Sites and Clinical Research Pilot Projects) by serving as the voice of the NJ ACE and promoting the sharing of lessons learned and best practices in the conduct of clinical research.

Two Clinical Research Program Sites were funded in 2012 and one in 2013. In addition, six Clinical Research Pilot Projects were funded in 2013. Program Site and Pilot Project grantees conduct clinical research projects that address the national priorities described in the Interagency Autism Coordinating Committee (IACC) Strategic Plan and, if applicable, grantees will also address the Healthy People 2020 autism-related objective addressed by the research project.

**Clinical Research Program Sites.** The Rutgers University Institute for Human Genetics was awarded a five year $2.2 million Program Site grant in June 2012 to focus on the genetics of Autism Spectrum Disorder (ASD). In December 2012 The University of Medicine and Dentistry of New Jersey (now Rutgers University)* was awarded a five year $2.1 million Program Site grant to study the neurobiological, molecular and genetic basis of autism and to explore novel therapies for affected individuals living in New Jersey. A third five year Program Site grant was awarded in June 2013 for $2.25 million to Children's Specialized Hospital in New Brunswick to develop a new ASD screening tool for culturally diverse families. To advance the goal of widespread data sharing among ASD researchers, investigators funded under the NJ ACE Program Sites grant program share their data via the NIH National Database for Autism Research (NDAR). NJACE Pilot Project grantees are encouraged, not required, to share data with NDAR. NDAR houses research data of all types (genetic, imaging, clinical assessment, etc.) from human subjects involved in ASD studies. It is expected that in the next several years, ASD data from more than 90% of new investigations will be available in or through NDAR. Submitting data to NDAR is a new requirement for Council's grantees and reflects the Council's priority in

optimizing clinical research activities in New Jersey and also contributing to the national autism clinical research effort. The New Jersey data will be made available to autism researchers from around the United States, highlighting the New Jersey effort, and similarly, national data will be available to New Jersey researchers to enhance their work and grant applications. Furthermore, participation in NDAR demonstrates that the funded sites are capable of meeting the National Institutes of Health (NIH) requirements and will help them develop stronger proposals for submission to the NIH and biomedical research foundations.

**Clinical Research Pilot Projects.** Enhancing the state's commitment to families affected by autism, Health Commissioner Mary E. O'Dowd joined the Governor's Council for Medical Research and Treatment of Autism in June 2013 to announce $4.5 million in research grants, including the Program Site grant to Children's Specialized Hospital as previously described. The Clinical Research Pilot Project grant awards were:

- $400,000 over 2 years to UMDNJ New Jersey Medical School (now Rutgers University-New Jersey Medical School)* to identify biomarkers that would identify a subtype of ASD.

- $399,846 over 2 years to Saint Peter's University Hospital in New Brunswick to examine the biological markers that can be useful in identifying children at risk for autism.

- $399,565 over 2 years to UMDNJ School of Osteopathic Medicine-SOM (now Rowan University-SOM)* in Stratford to examine the association between environmental pollutants and ASD.

- $399,336 over 2 years to UMDNJ New Jersey Medical School (now Rutgers University-New Jersey Medical School)* to evaluate perinatal risk factors such as parental age, maternal health status, premature birth and their influence on changes in ASD prevalence over time.

- $394,204 over 2 years to Rowan University in Glassboro to compare two interventions for preschool children with autism.

- $321,253 over 2 years to Rutgers University to research the transportation needs of people with ASD and develop policies, procedures and accommodations to improve the quality of life of those on the autism spectrum.

**Changes in the higher education infrastructure in New Jersey as a result of the Rutgers-University of Medicine and Dentistry of New Jersey (UMDNJ) merger, effective July 1, 2013.*

In June 2014 a total of seven two-year Clinical Research and Clinical Translational Research Pilot Projects were awarded grants:

- $400,000 to Rutgers University's Robert Wood Johnson Medical School in New Brunswick to study autism-linked stress at the cellular level and initial testing of therapeutic strategies.

- $400,000 to Rutgers University Robert Wood Johnson Medical School in Piscataway to study human stem cells from individuals with autism in order to determine metabolic abnormalities that may contribute to autism and have the potential to be reversed through the use of pharmaceuticals.

- $399,558 for the Center for Neurological and Neurodevelopment Health II in Gibbsboro to test for specific genetic abnormalities, assess for their clinical manifestations, and discover new biological causes of Autism Spectrum Disorder (ASD) and associated complications.

- $398,908 to Rutgers University Robert Wood Medical School in Piscataway to provide a new objective instrument that detects micro-movements present in social behaviors with the potential of leading to earlier diagnosis of autism, using the standardized structures of the Autism Diagnosis Observation Schedule (ADOS), a reliable instrument based on observation.

- $398,282 for Rutgers University's Center for Advanced Infrastructure & Transportation in Piscataway to perform an assessment to aid adults on the autism spectrum in finding safe, accessible and appropriate paratransit transportation services.

- $397,547 to Saint Peter's University Hospital in New Brunswick to analyze early electroencephalogram (EEG) features to predict risk of ASD and Related Disorders in Premature and low birth weight infants.

- $125,899 to William Paterson University in Wayne to examine imitation from video in children with ASD with a controlled experiment to support, or not, the proliferation of video and touchscreen based imitation interventions for ASD.

The Governor's Council has awarded nearly $25 million in autism grants since 2008.

## Research Funding in FY 15

Based on the availability of funds, Council will fund autism research in FY 15. An ad hoc committee of the Council has been charged with recommending the categories of research grants for funding in FY 15.

## Contact Information

**NJ Governor's Council for Medical Research and Treatment of Autism** PO Box 360, 225 East State Street, Second Floor, Trenton, New Jersey 08608

Email: NJGCA@doh.state.nj.us

Phone:
(609) 633-8740
or
(609) 943-5405
Fax:
(609) 943-4213

# Appendices

- Conference participants including research interests
- Conference evaluation

**APPENDIX**

# *Translational Research in Autism*
# Conference Participants
# April 9, 2014

## NJ Researchers

**Linda Brzustowicz, M.D., Professor**
Rutgers University, Department of Genetics
145 Bevier Rd., Piscataway, NJ 08854
Telephone number: 732-445-3331
Fax number: 732-445-1636
Email address: brzustowicz@dls.rutgers.edu
Research interests: We are investigating the genetic basis of autism spectrum disorder (ASD) using a family- based model. We have a particular interest in identifying genes and gene variants associated with language and communication behaviors associated with ASD.

**Gabriella D'Arcangelo Ph.D.**
Rutgers, the State University of New Jersey
604 Allison Road, room B323, Piscataway, NJ 08854
Telephone number: 732 445 2839
Fax number: 732 445 5870
Email address: darcangelo@dls.rutgers.edu
Research interests: Cellular and molecular mechanisms of mammalian brain development, and human developmental brain disorders

**Oana de Vinck-Baroody, DO (developmental pediatrician)**
Hackensack University Medical Center, Institute for Child Development
30 Prospect Avenue, Hackensack, NJ 07601
Telephone number: 551-996-8262
Fax number: 551-996-0808
Email address: odevinck-baroody@hackensackUMC.org
Research interests: media use in children with autism; transition planning in autism; exercise and behavior in children with autism; obesity and health issues in children with autism

**Emanuel DiCicco-Bloom, M.D.**
Professor, Departments of Neuroscience & Cell Biology and Pediatrics
Robert Wood Johnson Medical School, Rutgers, the State University of New Jersey
675 Hoes Lane, Piscataway, NJ 08854
Tel: 732-235-5381
Fax: 732-235-4990
Email address: diciccem@rwjms.rutgers.edu
Research interests: Our primary interest is to understand the neurobiological bases of autism, which we study at both the genetic and cellular levels. Our works employs animal models of autism associated

genes, such as Engrailed-2, as well as the use of human induced pluripotent stem cells to derive neuronal precursor cells that we study in cell cultures.

**Cecilia Feeley, Transportation Autism Project Manager**
Rutgers University, Center for Advanced Infrastructure and Transportation
100 Brett Road, Piscataway, NJ 08854
Telephone number: 848-445-3325
Fax number: 732-445-3325
Email address: cfeeley@rci.rutgers.edu
Research interests: The focus of my research is the transportation and mobility needs and issues for adults (ages 18 and over) on the autism spectrum. Issues that have I addressed professionally include barriers, accommodations, access, safety, training, assessments, quality of life and the impact that transportation can have on employment and housing. There has also been some work exploring the impacts technology can have on travel and mobility.

**Bonnie L. Firestein, Ph.D., Professor of Cell Biology and Neuroscience**
Rutgers University, 604 Allison Road, Piscataway, NJ 08854
Telephone number: 732-445-8045
Fax number: 732-445-8046
Email address: Firestein@biology.rutgers.edu
Research interests: My interest in autism is two-fold: 1) My lab studies the cellular mechanisms involved in neural circuit connectivity and how this connectivity may become aberrant in autism, and 2) We are interested in constructing an iPSC-derived neuronal system to study cellular phenotypes in autism.

**Judy Flax, Ph.D., Associate Research Professor**
Rutgers University, Department of Genetics
145 Bevier Rd., Piscataway, NJ 08854
Telephone number: 732-445-1224
Fax number: 732-445-1636
Email address: flax@dls.rutgers.edu
Research interests: We are investigating the genetic basis of autism spectrum disorder (ASD) using a family- based model. We have a particular interest in identifying genes and gene variants associated with language and communication behaviors associated with ASD.

**Jill Harris, Ph.D., Director of Program Development**
Children's Specialized Hospital
330 South Avenue, Fanwood, NJ 07023
Telephone number: 908-301-2525
Fax number: 908-301-2534
Email address: jharris@childrens-specialized.org
Research interests: Early screening for ASD; underserved populations; reducing barriers to diagnosis and services.

**Laura Henderson, MA, BCBA**
The Center for Neurological and Neurodevelopmental Health (CNNH), NeurAbilities
250 Haddonfield-Berlin Road; Suite 105; Gibbsboro, NJ 08026
Telephone number: 856-346-0005
Fax number: 856-784-1799

Email address: lhenderson@thecnnh.org
Research interests: Functional Behavior Assessments, Positive Behavior Supports in the classroom, Pivotal Response Training

**Lisa Huguenin, PhD**
Rutgers New Jersey Medical School
Address: 1017 Canal Rd Princeton NJ 08540
Telephone number: 609-915-5243
Email address: lzussman@hotmail.com
Research interests: Molecular characterization of an inflammatory subtype of autism

**Randye Huron, M.D.**
Institute for Child Development, Hackensack University Medical Center
30 Prospect Avenue, Hackensack NJ 07601
Telephone number: 551-996-5202
Fax number: 551-996-0808
Email address: rhuron@hackensackumc.org
Research interests: We were previously funded by the Clinical Enhancement Grants. We are involved in the prevalence studies of autism in New Jersey. We were involved in an epidemiological/environmental study regarding a correlation between toxic dump sites and the incidence of autism and other developmental disabilities (completed, but not yet published). We will be submitting a grant proposal application to the Governor's Council to study media use in the young autistic population.

**Yvette Janvier, M.D.**
Children's Specialized Hospital
94 Stevens Rd, Toms River, NJ
Telephone number: 732-797-3801
Email address: yjanvier@childrens-specialized.org
Research interests: Early screening for ASD; underserved populations; reducing barriers to diagnosis and services.

**Harumi Jyonouchi, M.D., Associate Professor**
NJMS-Rutgers, 185 South Orange Ave. F643 MSB, Pediatrics, Newark, NJ
Telephone number: 973-972-1414 (office), 973-919-3389 (cell)
Fax number: 973-972-6443
Email address: jyanouha@njms.rutgers.edu
Research interests: My current research interest is to address possible immune mediated inflammatory components affecting their behavioral and possibly cognitive activity in ASD subjects. I expect that this may be present in a subset of ASD subjects with fluctuating behavioral symptoms apparently triggered by immune insults such as microbial infection.

**MaryLou Kerwin, Ph.D., Professor**
Rowan University, 201 Mullica Hill Rd., Glassboro, NJ 08028
Telephone number: 856-256-4921
Fax number: 856-256-4892
Email address: Kerwin@rowan.edu

Research interests: I am interested in the treatment of autism spectrum disorder. Our current study is a treatment outcome study investigating parent-implemented treatments for preschool children with autism.

**Michael Lewis, Ph.D.**
University Distinguished Professor of Pediatrics and Psychiatry,
Director, Rutgers Robert Wood Johnson Medical School Autism Center,
Director, Institute for the Study of Child Development, Rutgers-RWJMS
Rutgers University, Robert Wood Johnson Medical School
89 French Street, Suite 1200, New Brunswick, NJ 08901
Telephone number: 732-235-7700
Email address: lewis@rwjms.rutgers.edu
Research interests: The development of Parent Friendly Apps for early diagnosis of ASD. Brain processes and Self Referential Behavior in TD and ASD Children. Facial Recognition and Intervention in Children with AS/ASD

**Andrea Lubin, Senior Researcher**
Voorhees Transportation Center, Rutgers University
33 Livingston Ave, New Brunswick NJ 08901
Telephone number: 848-932-2861
Fax number: 732-932-3714
Email address: annlubin@ejb.rutgers.edu
Research interests: I am a transportation policy researcher with Rutgers University, focusing on exploring the transport needs and issues of persons with disability, the elderly and other transportation disadvantaged population. I am currently working with principal investigator Cecilia Feeley on a Governor's council funded study that is examining the transport needs of adults on the autism spectrum in New Jersey.

**Audrey Mars, M.D.**
Medical Director, Hunterdon Medical Center Developmental Pediatric Associates, Clinical Associate Professor of Pediatrics, Voluntary staff, Rutgers- RWJMS
130 Route 31 N, Suite 500, Flemington, NJ 08822
Telephone number: 908-788-6650
Fax number: 908-788-6578
Email address: audreymars1@gmail.com
Research interests: Autism Prevalence: The New Jersey Autism Study, Genetic studies (PTEN), Early Identification of ASD

**Melanie McGackin, Coordinator of Training and Education**
Autism Family Services of NJ/ Family Support Center of NJ
35 Beaverson Blvd., Brick, NJ 08723
Telephone number: 732-528-8080
Fax number: 732-262-4373
Email address: Melanie.mcgackin@fscnj.org
Research interests: I am the Coordinator of Training and Education for Autism Family Services of New Jersey and the Family Support Center of New Jersey. Within my role at these agencies, I have the privilege to provide information, resources, and conduct trainings to benefit individuals with an autism diagnosis and their families. Through the project that I am currently working on, with principal

investigator Cecilia Feeley; I have the ability to connect our team with providers, state agencies, and families to help collect data surrounding transportation needs and barriers throughout the state of New Jersey.

**Heather McGowan, Graduate Student**
Child Health Institute of New Jersey
89 French Street, New Brunswick, NJ 09801
Telephone number: 201-388-9825
Email address: mcgowan326@gmail.com
Research interests: I am interested in synaptic development and function, and I am currently studying the effect of miRNA gene regulation on the synapse.

**James Millonig Ph.D., Associate Professor**
Robert Wood Johnson Medical School; Rutgers University
Center for Advanced Biotechnology and Medicine
Room 238, 679 Hoes Lane, Piscataway, NJ 08854
Telephone number: 732 235 3391
Fax number: 732 235 4850
Email address: Millonig@cabm.rutgers.edu
Research interests: Using stem cells to understand the neurodevelopmental basis of stem cells

**Mark Mintz, M.D.**
The Center for Neurological and Neurodevelopmental Health (CNNH), NeurAbilities
250 Haddonfield-Berlin Road; Suite 105; Gibbsboro, NJ 08026
Telephone number: 856-346-0005 x1101
Fax number: 856-784-1799
Email address: **mmintz@thecnnh.org**
Research interests: Clinical Phenotyping and Biological Causes of Autism and Related Disorders; Effective Medical and Non—Medical Treatments for Autism and Related Disorders; Epilepsy; ADHD and LearningDisorders; Neuro AIDS; Tics/TouretteDisorder

**Pnina Mitz, Ph.D.**
The Center for Neurological and Neurodevelopmental Health (CNNH), NeurAbilities
250 Haddonfield Berlin Rd, Suite 105, Gibbsboro, NJ 08026
Telephone number: 856-346-0005, x1014
Fax number:856-784-1799
Email address: pmintz@thecnnh.org
Research interests: Autism, special needs

**Zhiping Pang, Ph.D.,Assistant Professor**
Rutgers University Robert Wood Johnson Medical School
89 French Street, Room 3233
Telephone number: 732-235-8074
Email address: pangzh@rwjms.rutgers.edu
Research interests: My research focuses on synaptic regulation from stem cells to the brain. We use human neurons to model neuropsychiatric disorders.

**Nicole Pelliciari, Board Certified Behavior Analyst**
The Center for Neurological and Neurodevelopmental Health (CNNH), NeurAbilities
250 Haddonfield Berlin Road, Gibbsboro NJ 08026
Telephone number: 856=346-0005
Fax number: 856-784-1799
Email address: npelliciari@thecnnh.org
Research interests: Identifying successful variables in school and ABA therapeutic settings (ie: staff training, treatment hours, materials, etc)

**Nicholas M. Ponzio, Ph.D. Professor**
Rutgers University – New Jersey Medical School
Department of Pathology; 185 South Orange Avenue; Newark, NJ 07103
Telephone number: 973-972-5238
Fax number: 973-972-7293
Email address: ponzio@njms.rutgers.edu
Research interests: My research involves investigation of a prenatal mouse model of autism in which maternal immune stimulation during pregnancy results in alterations in fetal programming of the immune system, as well as the brain. As such, offspring of pregnant dams that receive immune stimulation exhibit a "pro-inflammatory" phenotype that predisposes them to respond more vigorously when given a post-natal immune challenge. This pro-inflammatory phenotype persists into adulthood, and is responsible for more severe immunopathology in mouse models of human disorders, such as cardiac disease and multiple sclerosis.

**Agnes Cushing-Ruby**, Clinical and Research Coordinator
NJMS-Rutgers, DOC 5100, 90 Bergen St., Newark, NJ 07103
Telephone number: 973-972-8120
Fax number: 973-972-5895
Email address: cushinag@njms.rutgers.edu
Research Interest: Autism Spectrum Disorder , Inflammatory Subtype

**T. Peter Stein, Ph.D.**
Rowan University – School of Osteopathic Medicine
Science Center, 2 Medical Center Drive, Stratford, NJ 08084
Telephone number: 856 566 6036
Fax number: 856 566 6042
Email address: tpstein@rowan.edu
Research interests: We are interested in determining whether there are any relationships between Autism and exposure to environmental pollutants.

**Harvey R. Weiss, PhD, Professor**
Rutgers Robert Wood Johnson Medical School
Department of Neuroscience and Cell Biology
675 Hoes Lane West, Piscataway, NJ 08854
Telephone number: 732-234-4626
Email address: hweiss@rutgers.edu

Research interests: I study the differential responses of cerebral metabolism to stimulatory and inhibitory neurotransmitters in the brains of control and autistic model animals. The role of mammalian target of rapamycin (mTOR) is now being examined in this regard.

**Venkat Venkataraman Ph.D., Assistant Professor**
Rowan School of Osteopathic Medicine
SC 220, Rowan-SOM, 2, Medical Center Drive, Stratford, NJ 08084
Telephone number: 856.448.3701
Email address: vvenkat2007@gmail.com
Research interests: I am looking into the relationship between blood-brain barrier and autoantibodies in the context of neuronal disorders.

**Barbie Zimmerman-Bier, M.D.**
The Children's Hospital at Saint Peter's University Hospital
254 Easton Avenue, New Brunswick, NJ 08903
Telephone number: 732-745-8600
Email address: zimmermanbier@gmail.com
Research interests: Our research is looking at very early and early markers for ASD and related disorders in a high risk cohort. We are focusing on premature and low birth weight infants and following placental markers, immunological markers, and eye tracking and motor movements markers that may indicate risk for ASD.

## Governors Council for Medical Research and Treatment of Autism

Caroline Eggerding M.D., Council Chairperson
B. Madeline Goldfarb MA, member, Chair, Planning Committee
Susan Evans, Ed.D., member
Liz Bell B.S., member
Martin T. Zanna, M.D., MPH, Acting Executive Director
Linda N. Boclair, M.Ed., MBA, Executive Assistant
Daphne Robinson, Ph.D., consultant

## Montclair State University, Center for Autism and Early Childhood Mental Health

Gerard Costa, Ph.D., Director and Senior Lecturer
Kaitlin Mulcahy, M.A., LPC, IMH-E® IV-Clinical Mentor, Associate Director
Karen Hood-Kasim, MPH, Interim Program Coordinator, NJ ACE Coordinating Center-MSU

## Montclair State University

Eden N. Kyse, Ph.D., Director, Ctr for Research & Evaluation on Education and Human Services (CREEHS)
Erin M. Bunger, MPH, Senior Research Associate, (CREEHS)
Haiyan Su, Ph.D., Assistant Professor, Mathematical Sciences
Jerry Alan Fails, Ph.D., Assistant Professor, Computer Science

## Speakers and discussion group leaders

Helen Tager-Flusberg, Ph.D. Professor, Department of Psychological; Brain Sciences Boston University; Director, Center for Autism Research Excellence

Alycia Halladay Ph.D., Senior Director of Research, Autism Speaks, NYC

Michael Lewis, Ph.D., University Distinguished Professor of Pediatrics and Psychiatry; Director of the Rutgers Robert Wood Johnson Medical School Autism Center; Director of the Institute for the Study of Child Development, Rutgers-RWJMS

Audrey Mars, M.D., Medical Director, Hunterdon Medical Center Developmental Pediatric Associates, Clinical Associate Professor of Pediatrics, Voluntary staff, Rutgers- RWJMS

James Millonig, Ph.D., Associate Professor, Rutgers RWJMS Neuroscience & Cell Biology

Thomas McCool, Ed.D., President Emeritus, Eden Autism Services.

## Additional State Representatives and Guests

Sandra Howell, Ph.D., Research Scientist, NJ Department of Health, Autism Registry

Nancy Scotto-Rosato, Ph.D., Research Scientist, NJ Department of Health, Autism Registry

Jonathan Sabin, Director, Office on Autism, NJ Department of Human Services

Carolyn M. Salafia MS M.D., Laboratory Head, Institute for Basic Research, Staten Island, NY

Eric London M.D., Director Autism Treatment Research Lab, NYS Institute for Basic Research in Developmental Disorders, Staten Island NY

Elizabeth M. Lennon, Ph.D., Department of Infant Development, NYS Institute for Basic Research in Developmental Disabilities, Staten Island, NY

## Conference Evaluation

Participants provided the following written comments indicating their reactions to the conference:

*"This meeting was greatly appreciated. It provided not only learning opportunities but reinforces motivation to continue working and looking for research opportunities.* Thank *you."*

*"Informative, interdisciplinary meeting."*

*"Enjoyed the day!"*

*"Thank you for the opportunity. It was a wonderful experience to gain knowledge from all the presentations and to share in the hope of making a difference in the autism community."*

*"A great and informative session even though I do not work in the biological sciences field (I am a social science researcher). A recommendation going forward, please start sessions later like 10am as it is difficult to travel here by 9am from further regions of the state. Thank you."*

*"Enjoyed the day and the lectures as well as meeting some new people. I would recommend having more opportunity for people to meet and get to know each other as well as for allowing individuals to choose the groups they participate in."*

*"Terrific Day! Next time perhaps include dialog across research projects, integrating their questions and findings."*

*"Really great to be together. Great idea to tackle the "big questions" currently being asked in autism. More time for collaboration in small groups – maybe form interdisciplinary groups so biomedical researchers are with clinicians etc."*

*"The day went well and presentations flowed smoothly. A nice size group that permitted opportunities to interact socially and begin networking in the future".*

*"I enjoyed the structure of the conference, engaging and informative. The DSM-5 discussion was eye opening both the clinical and research perspectives."*

*"I enjoyed the conference and meeting others working in the field".*

*"Great conference. I enjoyed the opportunity to speak to other researchers".*

*"I enjoyed Dr. Tager-Flusberg's presentation and the group discussions".*

*"Need to help build clinical/research infrastructure to apply translational work".*

*"I think it is a nice forum for us to communicate. It will be helpful to have more talks/discussions."*

*"Loved the talk by Helen Tager-Flusberg. She was able to describe by example what translational research can be. Next time how can we communicate to the lay public how important translational research is? I think it is misunderstood."*

*"Please have the next one at Rutgers (Piscataway)!"*

*"Very productive meeting. Minor point-can (some) meetings be in a more central location? This would really benefit those of us from south Jersey".*

*"This was an interesting meeting. I think one of the problems with translational research is that the definition of ASD has changed. It is much harder to get a homogenous group."*

*"This was my first time attending. Fascinating – Madeleine's personal touch and being "dumped" with MDs but had a great discussion with new ideas. Recommendation-next time in south Jersey and publish members earlier".*

*"More facilitation of more frequent, smaller meetings to discuss potential collaboration would be good. These meetings are nice but I don't think real collaborations get set up at these."*

*"We need a reward system that encourages interdisciplinary interactions."*

*"This was very informative and useful. Perhaps more time should be dedicated to intergroup discussions – after each group has met it would be a good time to come back together to discuss further."*

*"Breakout meetings are not adequately structured to be a learning experience. Goals unclear."*

*"You should make sure PowerPoints are given to all attendees."*

*"The panels should be given information about what they are going to be discussing".*

*"Maybe a parent panel".*

*"Everyone agrees about heterogeneity of ASD. Subgroup specific biomarkers or screening measures will be necessary for effective efficacy/outcome studies".*

*"Comment cards always suggest to me that you are looking for input. The focus was too narrow in focus. Neurology and genes are 2 pieces not all pieces. I would like to see broader parental input. Researchers would be well served to gain perspective from parents on issues and challenges".*

*"Discussion group should be better informed on what's most up dated. Integration between different systems should be encouraged. All future funding should encourage proactive engagement between clinical and basic sciences groups. None of the translation can be initiated without strong basic science. Recommend a "how to translate" workgroup for basic researchers in the state".*