



IACC Strategic Planning Workgroup Meeting

April 21, 2008

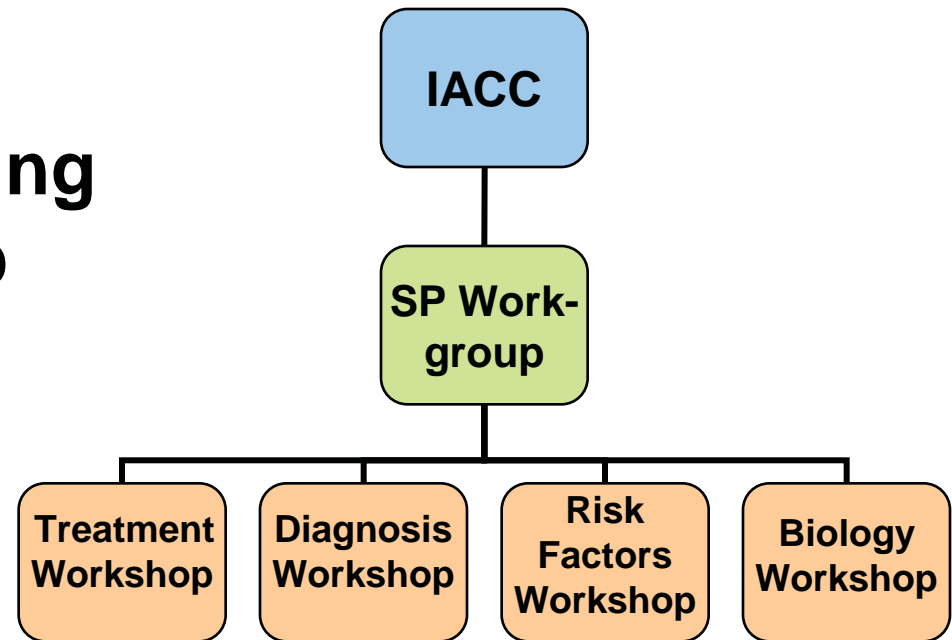
**Edwin Trevathan, M.D.
Centers for Disease Control and Prevention
IACC Meeting April 21, 2008**



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Structure for Strategic Plan

- **IACC**
- **Strategic Planning (SP) Workgroup**
- **Scientific Workshops**



All involve

- **Stakeholders**
- **NIMH Autism Team**

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Completed Steps

Strategic Planning Process Approved by IACC – Nov 07

Stakeholder RFI – Dec 07- Jan 08

Four Scientific Workshops Held – Jan 08

SP Workgroup Meeting – Feb 08

New SP Workgroup Formed by IACC – March 08

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SP WG Roster

- **Steve E. Hyman, M.D. (chair)** Harvard University
- **David Amaral, Ph.D.** University of California, Davis
- **Peter Bell MBA** Autism Speaks
- **Mark F. Blaxill MBA** Coalition for Safe Minds
- **Judith Cooper, Ph.D.** NIDCD
- **Geraldine Dawson, Ph.D.** Autism Speaks
- **Steve Eiken, M.A.** Thomson Healthcare
- **Daniel Geschwind, M.D., Ph.D.** UCLA
- **Martha Herbert, M.D., Ph.D.** Harvard Medical School
- **Alice Kau, Ph.D.** NICHD
- **Catherine Lord, Ph.D.** University of Michigan
- **David Mandell, Sc.D.** University of Pennsylvania
- **Prisca Chen Marvin, J.D.**
- **Sam Odom, Ph.D.** University of North Carolina
- **Isaac Pessah, Ph.D.** University of California, Davis
- **Denise D. Resnik** SARRC
- **Stephen Shore, Ed.D** IACC Public Member
- **V. Fan Tait, M.D.** American Academy of Pediatrics
- **Edwin Trevathan, M.D., MPH** CDC
- **Lucille Zeph, Ed. D.** University of Maine
- **Andrew Zimmerman, M.D.** Kennedy Krieger Institute

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Public Participation

- Public registrants could listen to the meeting via teleconference link and view slides on the Internet
- 198 registered
- 121 participated
- Average length of call = 229 minutes
- Two workgroup members and some IACC members participated virtually and had the capability to speak and be heard

Values

- **Sense of Urgency** – We will focus on what steps we can take now to be responsive to the needs of individuals and families affected by ASD.
- **Spirit of Collaboration** – We will treat others with respect, listen to diverse views with open minds, and foster discussions where participants can comfortably offer opposing opinions.
- **Consumer-focused** – We will focus on making a difference in the lives of people affected by ASD, including individuals with ASD, their families, medical practitioners, educators, and scientists.

Values

- **Excellence** – We will pursue basic and clinical research of the highest quality to protect the safety and advance the best interests of those affected by ASD.
- **Partnerships in Action** – We will value cross-disciplinary approaches, data sharing, teamwork, and partnerships with clearly defined roles and responsibilities.
- **Accountability** – We will develop SMART (specific, measurable, achievable, realistic, and time-bound) research objectives aligned with funding priorities and develop systems for evaluation and course corrections.

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Framework for Initiatives

- I. **WHEN** should I be concerned about my child's development? (7)
- II. **HOW** can I understand what is happening to my child? (9)
- III. **WHAT** caused this to happen and can this be prevented? (7)
- IV. **WHICH** treatments will help my child? (9)
- V. **WHERE** can I turn for services? (4)
- VI. **WHAT** does the future hold? (5)

Goals for Workgroup

Review the 2007 ASD research funding portfolio

Prioritize research initiatives within the six question framework

Discuss budgetary requirements of the strategic plan

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NIH FY 2007 Autism Portfolio

- \$127 million in autism grants, contracts, intramural research projects.
- Additional \$3.9 million invested in National Database for Autism Research (NDAR) in FY07.
- NIH Autism Coordinating Committee (ACC) categorized diverse portfolio of research activities into five broad research areas with subcategories.
- Assigned each research activity and associated funding to one research area and subcategory based on its primary aims and objectives.

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Research Areas and Corresponding Subcategories

- **Biology**
 - *Subcategories:* Clinical Neuroscience, Basic Neuroscience, and Biological Systems
- **Treatment**
 - *Subcategories:* Psychopharmacology, Biomedical, Behavioral/Psychosocial, Services Research, and Biomarkers for Treatment Response
- **Diagnosis**
 - *Subcategories:* Instrument Development, Early Identification, Characterization, Incidence/Prevalence
- **Risk Factors**
 - *Subcategories:* Genetics/Genomics, Environmental Influences and Gene x Environment Interplay, Mechanisms and Model Systems of Environmental Influences, and Psychosocial
- **Other**
 - *Subcategories:* Research Resources (e.g., data systems, repositories of biomaterials), Education and Dissemination, and Other

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FY 2007 NIH Autism Portfolio

Total Autism Funding by Research Area and Subcategory

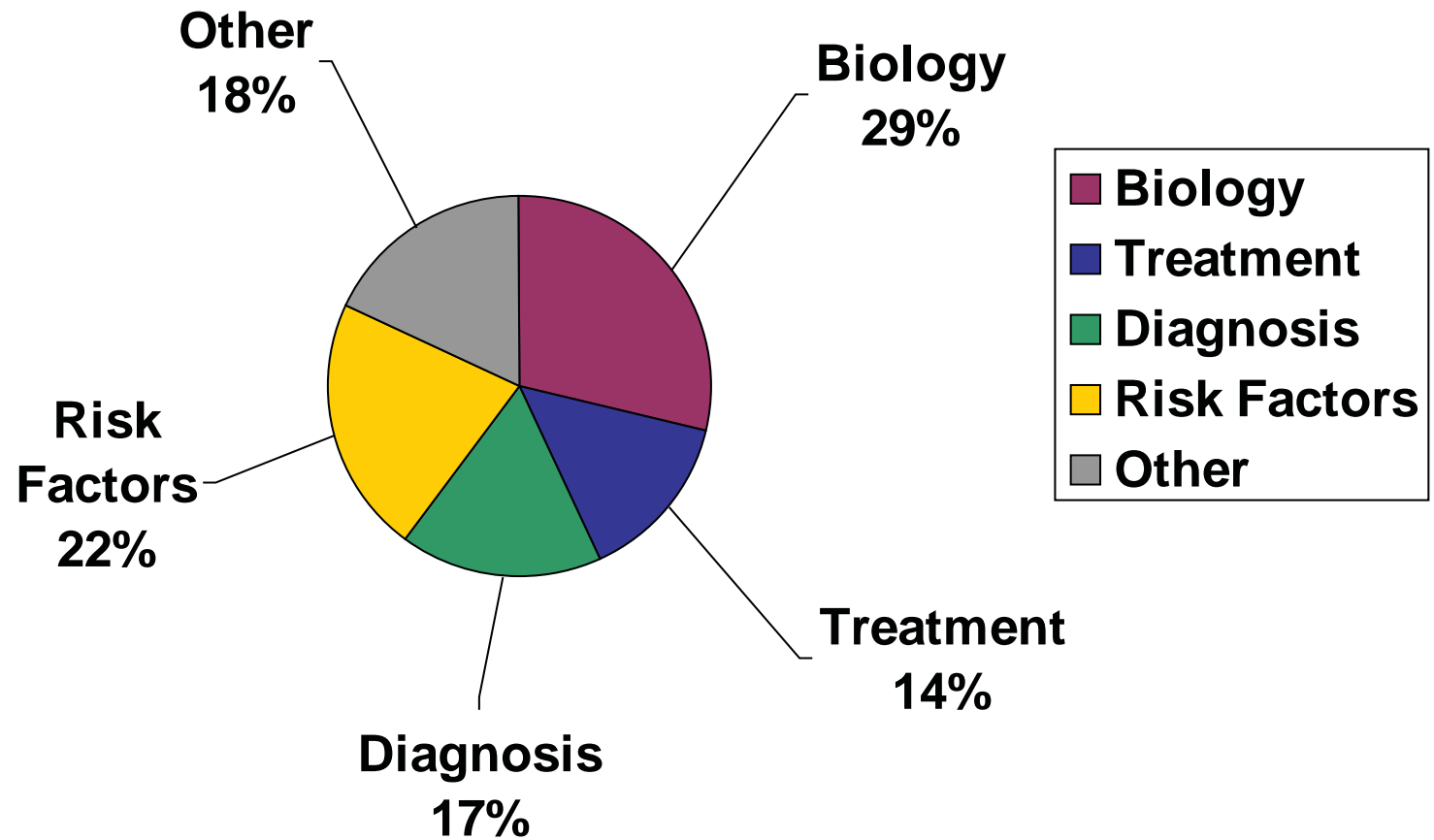
Subcategory	Research Area					
	Biology	Treatment	Diagnosis	Risk Factors	Other	
Clinical Neuroscience	\$22,407,705					
Basic Neuroscience	\$13,517,497					
Biological Systems	\$542,423					
Biology Subtotal	\$36,467,625					
Psychopharmacology		\$4,963,704				
Biomedical		\$1,201,569				
Behavioral/Psychosocial		\$10,275,206				
Services Research		\$1,576,656				
Biomarkers for Treatment Response		\$161,869				
Treatment Subtotal		\$18,179,004				
Instrument Development			\$1,601,155			
Early Identification			\$2,507,980			
Characterization			\$15,704,246			
Incidence/Prevalence			\$1,057,328			
Diagnosis Subtotal			\$20,870,709			
Genetics/Genomics				\$20,670,059		
Environmental Influences and Gene X Environment Interplay				\$6,672,090		
Mechanisms and Model Systems of Environmental Influences				\$356,750		
Psychosocial				\$644,943		
Risk Factors Subtotal				\$28,343,842		
Research Resources					\$18,012,053	
Education and Dissemination					\$4,149,842	
Other					\$820,660	
Other Subtotal					\$22,982,555	
Grand Total	\$36,467,625	\$18,179,004	\$20,870,709	\$28,343,842	\$22,982,555	\$126,843,735

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FY 2007 NIH Autism Portfolio

Percentage of Total Autism Funding (\$126,843,735)
by Research Area



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FY 2007 Autism Portfolio

Total Autism Funding* by Organization and Research Area

FY 2007 Autism Portfolio						
Total Autism Funding by Organization and Research Area						
Organization	Research Area					Total
	Biology	Treatment	Diagnosis	Risk Factors	Other	
Autism Speaks ¹	\$8,646,371	\$5,592,048	\$2,585,389	\$7,491,421	\$3,420,374	\$27,735,603
Centers for Disease Control and Prevention	\$0	\$0	\$8,360,000	\$6,920,000	\$0	\$15,280,000
Department of Defense ²	\$2,308,081	\$1,715,101	\$336,437	\$1,580,834	\$0	\$5,940,453
National Institutes of Health	\$36,467,625	\$18,179,004	\$20,870,709	\$28,343,842	\$22,982,555	\$126,843,735
The Simons Foundation	\$2,860,722	\$0	\$551,856	\$8,719,276	\$65,000	\$12,196,854
Grand Total	\$50,282,799	\$25,486,153	\$32,704,391	\$53,055,373	\$26,467,929	\$187,996,645

¹ Biology includes Autism Tissue Program (ATP), Treatment includes Autism Treatment Network (ATN) and Clinical Trials Network (CTN), Risk Factors includes Autism Genetics Project (AGP) and Autism Genetics Resource Exchange (AGRE), and Other includes Interactive Autism Network (IAN), conferences, training, and informatics.

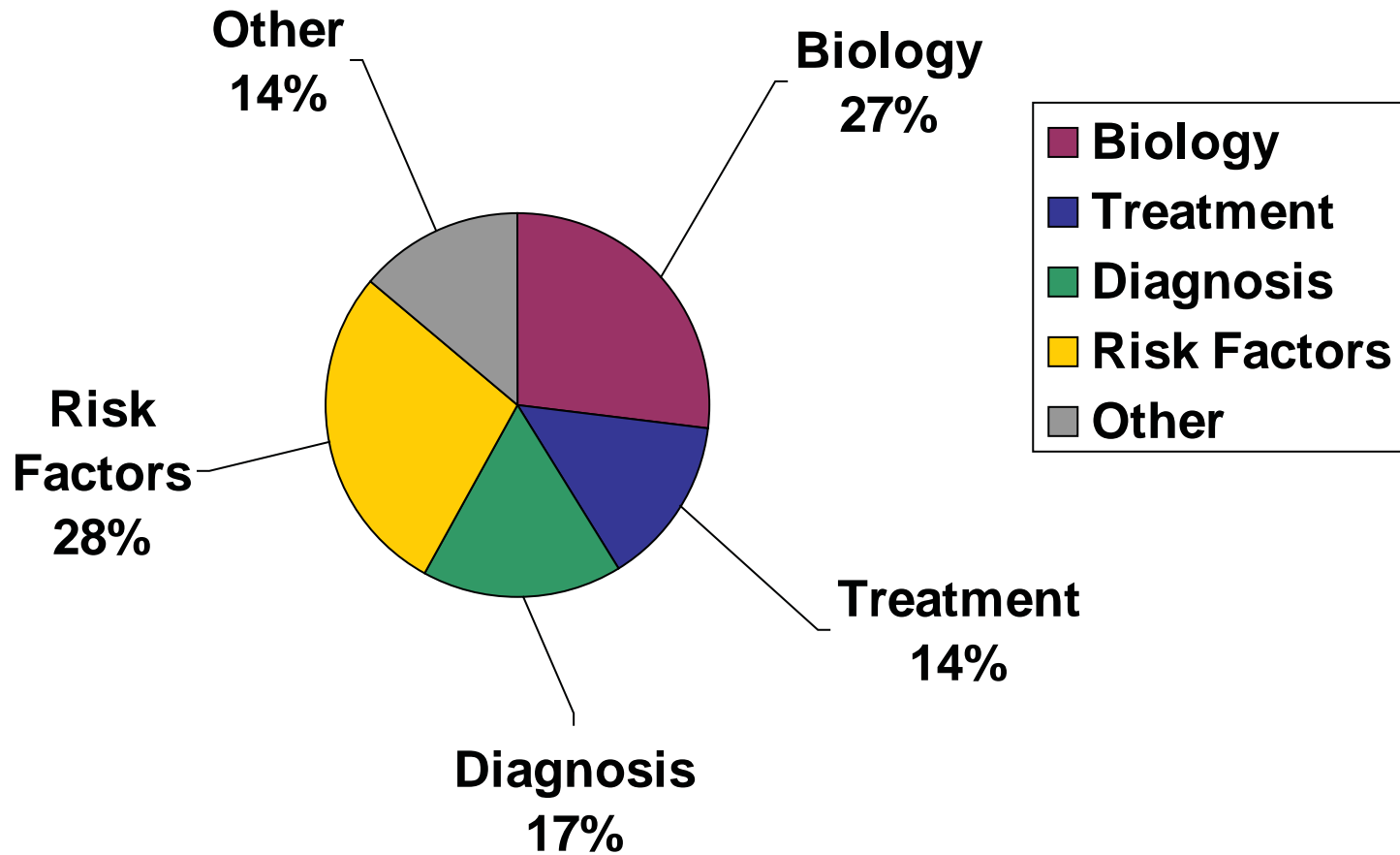
² The amounts for Biology and Diagnosis do not include all awards because some awards are not final. The amounts listed for Treatment and Risk Factors are tentative because they have not been finalized and awarded.

*NOTE: Funding data reflect investments from Autism Speaks, CDC, DoD, NIH and The Simons Foundation.

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FY 2007 Autism Portfolio

Percentage of Total Autism Funding* (\$187,996, 645)
by Research Area



*NOTE: Funding data reflect investments from Autism Speaks, CDC, DoD, NIH, and The Simons Foundation.

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Prioritization of Initiatives

- 1. Discussed and developed criteria for prioritization**
- 2. Prioritized within the six question domains to ensure coverage in each area**
- 3. Each workgroup member selected highest priority initiatives and distributed 100 points amongst choices**

Prioritization of Initiatives

- 4. Results of workgroup scoring were tallied**
- 5. Results of prioritization reviewed**
- 6. Prioritized initiatives forwarded to IACC**

SP WG Comments

- Emphasize to IACC that this is a “work–in–progress”
- Would like to have a vision, mission and aspirational goals to provide overall structure for WG deliberations
- Took a middle-ground position that more work needs to be done but doing initial prioritization will move things forward

**I. WHEN SHOULD I BE CONCERNED ABOUT
MY CHILD'S DEVELOPMENT?**

515 (12) I.C.2. Identify Relevant Phenotypes that Relate to Etiology, Symptom Presentation, and Outcome

360 (10) I.A.2. Collaborative Development of Streamlined Screening/Diagnostic Approaches

359 (12) I.A.3. Screening and Diagnostic Instruments in Underrepresented Populations

276 (11) I.C.1. The Development of Improved Categorical and Dimensional Measures of ASD

234 (9) I.B.2. Characterizing and Improving the Diagnostic Process in the Community

234 (8) I.A.1. Predictive Validity of Existing Screens in Community Setting

120 (3) I.B.1. Evaluating Diagnostic Criteria/Approaches

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II. HOW CAN I UNDERSTAND WHAT IS HAPPENING TO MY CHILD?

- 381 (10) II.A.3. Role of Immune and Infectious Factors in the Pathogenesis of Autism – Human and Animal Studies**
- 333 (12) II.A.4. Postmortem Brain and Tissue Acquisition Initiative**
- 297 (11) II.B.2. Developing Biomarkers for Autism**
- 296 (9) II.A.1. Multidisciplinary Longitudinal Study of Infants with Autism Before Age Three**
- 263 (9) II.A.2. Understanding Mechanisms of Neuroplasticity in Autism**
- 220 (7) II.B.1. Gender Differences in the Biological Features of Autism**
- 145 (5) II.B.3. Gene-based Phenotyping and Cognitive Neuroscience**
- 108 (3) II.A.5. Identification of Large-Scale Neural Systems Whose Function is Altered in Pre-adolescent Autism**
- 45 (2) II.B.4. New Paradigm for Clinical Genetic Evaluation and Subsequent Diagnosis**

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III. WHAT CAUSED THIS TO HAPPEN AND HOW THIS BE PREVENTED?

- 652 (17) III.B.3. Analysis of Mechanisms Underlying the Interplay of Genetic and Environmental Factors**
- 373 (11) III.B.5. Develop Resources for Appropriate Control and Comparison Groups for Biological, Genetic and Other Studies of ASD**
- 364 (14) III.B.2. Studies of Risk Factor Exposures From Pre-Conception to Early Postnatal Life**
- 230 (8) III.B.4. Methods Development for Biologic Exposures / Biomarkers**
- 208 (5) III.A.1. Large-scale Resource of Genomic Data on Autism Spectrum Disorders**
- 148 (5) III.B.1. Risk Factor Studies in Other Special**
- 124 (5) II.A.2. Informing the Genetics and Neurobiology of ASD Based on New Heritable Phenotypes Populations**

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IV. WHICH TREATMENTS WILL HELP MY CHILD?

- 455 (13) IV.A.1. Interventions for Older Children and Adults with ASD**
- 313 (9) IV.B.2. Investigation of Efficacy and Safety of Commonly Used and Untested Treatments for ASD**
- 285 (9) IV.A.2. Intervention and Prevention Approaches for Infants and Toddlers at Risk for Autism**
- 252 (7) IV.B.4. Fast Track Mechanisms to Facilitate Translational Treatment Research**
- 211 (6) IV.B.3. Animal Models and Cellular Systems for Developing Treatments for Autism**
- 195 (9) IV.B.1. Role of Co-morbidity in ASD Treatment**
- 140 (6) IV.A.3. Efficacy Trials for Comprehensive Intervention Models for Individual with ASD Across Ages**
- 98 (4) IV.C.2. Novel Treatments of Core Symptoms**
- 48 (2) IV.C.1. Identification of Biomarkers to Guide Treatment Selection and Evaluation of Treatment Outcome**

V. WHERE CAN I TURN FOR SERVICES?

623 (15) V.A.2. Identify and Evaluate Models of Effective Dissemination of Evidence-Based Practices (EBP) into Community Programs

568 (13) V.A.3. Evaluation of Community-Based Intervention Models Informed by Multi-Disciplinary Best Practices

454 (11) V.A.4. Cost-Outcome Studies of Intervention Models for People with Autism Spectrum Disorder (ASD)

445 (10) V.A.1. State of the States for Individuals with Autism Spectrum Disorder (ASD)

VI. WHAT DOES THE FUTURE HOLD?

- 487 (12) VI.A.2. Improved Identification and Characterization of Autism in Adulthood**
- 453 (13) VI.C.2. Develop Resources to Coordinate Large Population-Based ASD Initiatives (NDAR, CDC, NIH), IAN, State Registries)**
- 436 (9) VI.A.1. Understanding Developmental Trajectories of Children and Families Affected by ASD**
- 433 (9) VI.B.1. Enhance Tracking of ASD Prevalence in Children and Adolescents**
- 291 (7) VI.C.1. Merging and Analyzing Administrative Databases Relevant to Diagnosis, Course, Interventions, and Long-Term Outcomes**

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Comments on Results

- Some individual initiatives scored noticeably higher than the others in the same question domain
- Overlap areas are instructive in that they highlight areas of general interest, e.g. biomarkers- consolidation of initiatives can now be done
- Different opinions about scoring within question bins versus across question bins

Budgetary Requirements

- Treatment funding % was relatively smaller - but other areas inform treatment such as diagnosis (early ID), or biological studies of mechanisms
- “Don’t starve one area to feed another”
- Could reappportion within a funding domain, e.g. risk factors – increase environment, decrease genetics

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Budgetary Requirements

- Need more clinical trials infrastructure – share with other development disorders?
 - Efforts by Autism Speaks CTN and ATN noted
- Need for fast track mechanisms for high risk, high yield or n of 1 studies
- Some studies are more expensive to do than others so just looking at % can be misleading
- % in areas not as important as putting funds toward highest priorities

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Usefulness of SP

- Identify priorities so that partnerships between private and government groups will facilitate achieving the goals
- Develop RFAs or special emphasis panels
- Enable ASD initiatives to be funded that may be out of priority score order
- Attract excellent researchers to ASD research – SP makes a strong case for building opportunities and stimulating quality research



Today's IACC Discussion

- IACC to review draft vision, mission and aspirational goals for strategic plan
- Review and discuss a draft template for the strategic plan
- Approve further development of a SWOT (Strengths, Weaknesses, Opportunities, Threats) analysis of the comprehensive funding portfolio and 41 research initiatives



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Next Steps

- Review input from Town Hall Meeting
- Develop short and long term objectives with measurable outcomes
- Refine prioritization and consolidate research initiatives
- Consider infrastructure/mechanism needs