Novel Developments to Address Methodological Issues in Autism Spectrum Disorder

ISCTM  February 2016
Larry Alphs, MD, PhD
Considerations Related to Autism

- About 1 percent of the world population has autism spectrum disorder. (CDC, 2014)
- Prevalence in the United States is estimated at 1 in 68 births. (CDC, 2014)
- More than 3.5 million Americans live with an autism spectrum disorder. (Buescher et al., 2014)
- Prevalence of autism in U.S. children increased by 119.4 percent from 2000 (1 in 150) to 2010 (1 in 68). (CDC, 2014) Autism is the fastest-growing developmental disability. (CDC, 2008)
- Prevalence has increased by 6-15 percent each year from 2002 to 2010. (Based on biennial numbers from the CDC)
- Autism services cost U.S. citizens $236-262 billion annually. (Buescher et al., 2014)
- A majority of costs in the U.S. are in adult services – $175-196 billion, compared to $61-66 billion for children. (Buescher et al., 2014)
- Cost of lifelong care can be reduced by 2/3 with early diagnosis and intervention. (Autism. 2007 Sep;11(5):453-63; The economic consequences of autistic spectrum disorder among children in a Swedish municipality. Järbrink K1.)
Autism Spectrum Disorder: Pulled from the Headlines

JAMA February 16, 2016: The US Preventive Services Task Force (USPSTF) recently released a recommendation on screening for autism spectrum disorder (ASD).¹ In truth, this was a non-recommendation that stands in stark contrast to clinician groups, including the American Academy of Pediatrics, that recommend systematic screening for ASD at ages 18 and 24 months.² Finding insufficient evidence that screening for ASD changes outcomes in 18-to 30-month-old children, the USPSTF landed in a gray zone of neither supporting nor opposing screening. Instead, they deferred decision making to families and clinicians.
Autism Speaks Initiatives

Global Autism Public Health Initiative

Southeast European
South Asian
Gulf Coast Countries
Goals of Session

• Discuss the heterogeneity of the clinical presentation of ASD
• Discuss the challenges of identifying a suitable population for clinical trials
• Identify gaps in finding meaningful endpoints that track important symptoms and outcomes
• Identify regulatory considerations related to clinical trials for ASD
• Identify opportunities for stakeholder to partner to advance ASD research
## Agenda

<table>
<thead>
<tr>
<th>TOPIC</th>
<th>SPEAKER</th>
</tr>
</thead>
<tbody>
<tr>
<td>Challenges to patient identification in clinical trials for the treatment of Autism-Spectrum Disorder</td>
<td>L Gault</td>
</tr>
<tr>
<td><em>Lessons Learned: Development of a Novel GABA-B Receptor Agonist for ASD and Fragile X</em></td>
<td>P Wang</td>
</tr>
<tr>
<td>Autism Spectrum Disorder: Outcome Measures and Potential Biomarkers</td>
<td>G Pandina</td>
</tr>
<tr>
<td>The Translational Neurobiology of Autism</td>
<td>D Murphy</td>
</tr>
<tr>
<td>Regulatory Considerations When Conducting Clinical Trials for the Treatment of Autism Spectrum Disorder</td>
<td>T Farchione</td>
</tr>
<tr>
<td>Panel Discussion: How do we partner with stakeholders to move autism research forward, and facilitate biomarker validation and endpoint development?</td>
<td>S Zukin</td>
</tr>
</tbody>
</table>