



Novel Developments to Address Methodological Issues in Autism Spectrum Disorder

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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



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

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