Autism Spectrum Disorder Working Group

Working Group Session – 15th Annual Scientific Meeting- 21 February 2019, Washington DC

Report

Chairs: Celso Arango, Tiffany Farchione, Valentina Mantua

	2019 Autumn Conference includes Joint Day with ECNP Agenda 5-7 September 2019	
	6 September ASD Session	
13:45	Session 3**: Drug development for autism spectrum disorder: challenges and opportunities** *There is a clear unmet need in the availability of medicinal products for the treatment of Autism Spectrum Disorder (ASD). Despite the great amount of scientific investigation into the neurodevelopmental and biological causes of ASD, research has failed in delivering a therapy. The session will follow the drug development process addressing the areas of major interest from molecular targets and pathways to biomarkers for population stratification and outcome assessments. The main trial design methodology challenges will be addressed, and this would feed regulatory considerations and discussion.*	**Valentina Mantua, Tiffany Farchione, Celso Arango**
13:45-13:55	Intro	**Tiffany Farchione**
13:55-14:15	Personalized Medicine in Autism Spectrum Disorders – AIMS2 Trials	**Declan Murphy**
14:15-14:25	Discussion	
14:25-14:45	Biomarkers in Autism Spectrum Disorders – ABC-CT	**Jamie McPartland**
14:45-14:55	Discussion	
14:55-15:10	Break	
15:10-15:30	Technology platforms	**Gahan Pandina**
15:30-15:40	Discussion	
15:40-16:00	Methodological Challenges in Designing Clinical Trials for Autism Spectrum Disorders	**Evdokia Anagnostou**
16:00-16:10	Discussion	
16:10-16:30	Overarching/Integrated/Unanswered questions	**Jeremy Veenstra-Vanderweele**
16:30-17.00	Regulatory Comment	**Tiffany Farchione Valentina Mantua**
17.00-17:20	Panel Discussion (including members of family/patients' associations)	
17:20-17:40	Discussion	
17:40-17:45	Summary Conclusions	**Celso Arango**

17:30-19:00	Poster Session/ Reception	

Agenda Item 1: Summary of Working Group activity – for information

Agenda Item 2: Preparatory discussion on the upcoming Session

The Chairs shared the agenda with the group.

The group decided to have regular teleconferences with speakers to make sure that the presentations cover all topics identified with no overlap. This is particularly relevant for the first two talks on biomarker consortia, commonalities among different groups as well as methodological challenges should be presented.

Evdokia Anagnostou mentioned that the peculiarities of autism research should be emphasized such as role of parent/caregiver report, observation of changes in behavior and symptoms, persistence of acquired skills and abilities.

Identifying subgroups, surrogates, placebo response, maintenance of effect, timing of intervention and window of observation emerged as desirable topics for presentation/discussion.

Gahan Pandina is hoping to give a broad perspective on the role of digital medicine in autism research and drug development.

The group agreed to include representatives from a patient association in the panel for discussion

Agenda Item 3: Publication.

The second deliverable and outcome of the WG will be a Special Issue of *European Neuropsychopharmachology*, the journal of the European College of Neuropsychopharmacology. This possibility has been discussed and agreed with the Editor in Chief.

Tiffany Farchione, Celso Arango and Valentina Mantua will be editors for this issue.

The working title of the Special Issue is: Opportunities and Challenges in Drug Development for ASD: Product of ISCTM/ECNP Joint Working Group

The structure of the publication will include an opening Editorial by the President of the ECNP summarizing the context and objectives of the Special Issue.

A Main Paper will follow, highlighting the challenges drug development in Autism Spectrum Disorder and the proposed way forward.

The Chairs will approach James McCracken as first Author of the Main Paper; the deadline for delivering the first draft will be September 6th (WG session during the Autumn Meeting).

The Main Paper will be followed by 5 to 7 Commentaries.

The group discussed ideas and topics for both the Main Paper and the Commentaries.

During the discussion, the following participants volunteered to write a Commentary:

Gahan Pandina: The role of Digital Medicine

Janice Smith: Endpoints

Cristan Farmer: Inclusion of low IQ individuals

Ron Marcus and Dragana Bugarski-Kirola: Comorbidities and behavioural issues

One Commentary will focus on regulatory issues and another on patients/families' perspective on meaningful endpoints

Celso Arango proposed Stephen Leucht to comment on placebo response in this population (what is NOT unique to placebo and transdiagnostical challenges e.g. commonalities with schizophrenia)

The following WG session will discuss the publication and the first draft received will be commented with the group and the Authors of the Commentaries.