

6th International Scientific Conference 2018 Germany

The Angelman Syndrome Conference will be held in Hamburg, Germany

Location Hyperion Hotel Hamburg Amsinckstraße 39 20097 Hamburg https://www.h-hotels.com/de/hyperion/hotels/hyperion-hotel-hamburg

Scientific Symposium

The scientific symposium on Friday is planned for expert audience (researchers and scientists) but parents are welcome to attend.

Arrival Thursday, October 11 (evening), 2018

Symposium Friday – Saturday, October 12 – 13, 2018

Departure Saturday, October 13 evening or Sunday, October 14 morning

Parents Day: Saturday, October 13, 2018

On saturday a program for general audience is planned.



Executive organization: Angelman e.V. Germany www.angelman.de

Tentative Program

Thursday, October 11

- 19:00 Dinner
- 20:00 Plenary Lecture, chair:M. Scheffner and H. Sitte (ASA SAB) and B. Gerlach (Angelman e.V Deutschland)
- 21:00 Get-together

Friday, October 12 (expert audience)

- 8:30 Opening, chair: M. Scheffner and H. Sitte
- 8:35 Plenary speaker: Y. Elgersma
- 9:15 Coffee/tea break
- Session 1 Basic Research
 - 9:45 Speaker 1: S. Chamberlain
 - 10:15 Speaker 2: B. Distel
 - 10:45 Speaker 3: S. Russo
 - 11:15 Speaker 4: B. Philpot
 - 11:45 Lunch and scientific poster session
- Session 2 Clinical (Transmitters/Genetics)
 - 13:00 Speaker 1: K. Egawa
 - 13:30 Speaker 2: C. Kannegießer-Leitner
 - 14:00 Speaker 3: B. Horsthemke
 - 14:30 Coffee/tea break and scientific poster session

Session 3 Pharma

- 15:15 Introduction, chair: M. Scheffner and H. Sitte
- **15:20** Speaker 1: Ed Weeber with J. Daily Agilis Biotherapeutics
- **15:50** Speaker 2: E. Schneider IONIS Pharmaceuticals, Inc.and E. McNeil, Biogen
- **16:30** Speaker 3: OVID Gaboxadol trial *(request sent)*
- **17:30** Refreshments and scientific poster session
- **19:00** Leaving for cultural program
- 20:00 Dinner & cultural program open bar

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Tentative Program

Saturday, October 13 (general audience)

- Opening chair: M. Scheffner and H. Sitte 8:25
- 8:30 Speaker 1: Y. Elgersma
- 9:00 Speaker 2: K. Egawa or S. Chamberlain
- 9:30 Speaker 3: C. Kannegießer-Leitner

10:00 Coffee/tea break

- 10:30 Speaker 4: E. Weeber with J.Daily, Agilis Biotherapeutics
- Speaker 5: E. Schneider IONIS Pharmaceuticals, Inc. 11:10 and E. McNeil, Biogen
- Speaker 6: OVID Gaboxadol trial (request sent)
- 11:50
- 12:30 Lunch
- 14:00 Talks ASA Grant Winner 2016 & 2018 (S. Russo, B. Philpot and B.Distel)
- 15:30 Round table Scientists/Pharmas and ASA Board Meeting
- 17:00 Departure

Scientific Advisory Board (SAB)



Prof. Dr. rer. nat. Martin Scheffner University of Konstanz, Germany

Due to the notion that Angelman Syndrome (AS) is - by definition - a rare disorder, AS has for a long time not received the attention that would be required to

make a difference in both the academic and pharmaceutical worlds. In the past ten years, this has changed, mainly due to the spirit and dedication of families with AS children to bring together scientists from basic and clinically oriented research areas. The most recent highlight of such gatherings has been the biennial international scientific conference of the Angelman Syndrome Alliance (ASA), which will be held in Germany in 2018. In the ASA conference, distinguished scientists and clinicians/ pediatricians working on different AS-related aspects will present and discuss newest findings and developments, including novel therapeutic strategies and their potential pitfalls. Needless to say that that such discussions are invaluable for the progress of AS research with respect to both elucidation of the mechanisms underlying AS and the development of treatment strategies. The participation of parent organizations from all over the world is an important feature of the ASA conference. Besides the notion that the regular contact with AS families provides AS researchers with a particular motivation, this will ensure that the progress made is communicated to AS families.



Univ. Prof. Dr. Harald Sitte Medical University of Vienna, Austria

poster session

presented by parents

A strong base for networking activities is necessary to stay up to date and in touch with all the researchers that have one common goal in mind: to understand how

the Angelman Syndrome essentially works and what to do to alleviate its symptoms for the patients and their families. In this regard, a biennial European meeting is of utmost relevance for all researchers interested in the Angelman Syndrome and adjacent rare disorders.





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Speakers

Specific topics will be announced ASAP



Ype Elgersma, PhD Professor of Molecular Neuroscience Scientific director ENCORE Expertise Centre for Neurodevelopmental Disorders Department of Neuroscience, Erasmus MC, Holland



Prof. Dr. **Ben Distel** Associate professor (UHD), Biochemistry and molecular biology



Stormy J. Chamberlain

Chair, Scientific Advisory Committee Angelman Syndrome Foundation, Inc. Department of Genetics and Genome Sciences UConn Health, USA



Prof. **Edwin J. Weeber**, PhD Molecular Pharmacology and Physiology CSO Byrd Alzheimer's Institute, USA *with J. Daily (see below)*



Kiyoshi Egawa Pediatric Department, Hokkaido University Graduate School od Medicine, Japan

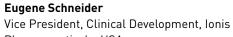


J. Daily,
Agilis Biotherapeuthics, USA
→ Scheduled clinical trials for gene therapy based on adenovirus in 2019



Ben Philpot PhD Philpot Lab, University of North Carolina, USA





- Pharmaceuticals, USA with Elizabeth McNeil (see below) → Natural history study efforts in An-
- gelman Syndrome and the scientific rationale for therapy with antisense oligonucleotides

Elizabeth McNeil Biogen USA

→ Natural history study efforts in Angelman Syndrome and the scientific rationale for therapy with antisense oligonucleotides

Dr. **Christel Kannegießer-Leitner** Researchgroup Angelman Verein e.V., Germany

→ Symptomatic treatment of neurotransmitters with GABAergic substances, presentation of the data collection of the German Angelman Association

Dr. **Silvia Russo** PhD Responsabile Sezione Genetica Malattie Rare; Usso; Istituto Auxologico Italiano; Laboratorio Biologia Molecolare, Italy



Bernhard Horsthemke PhD Professor and Chair Institute of Human Genetics Essen, Germany, University Hospital





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F	ees	Conference (payment to Angelman e.V. / account see below)			
		Thursday – Saturday**:	315 Euro*	** ASA Members will receive a	
		Friday – Saturday**:	270 Euro*	discount of EUR 100.00 on each conference participant. This discount is limited to 2 ASA members of each country. Additional members will pay the normal day rates.	
		Friday only:	225 Euro*		
		Saturday only:	195 _{Euro*}		
		Saturday only for members 95 Euro*		* Angelman kids are very welcome at the conference. Angelman children will have free entry. Please be aware that no special	
		* Meals and drinks included.		children program or assistance will be available.	
Accommodat	tion	Hotel (paid directly to the ho	tel)	Preferential rates have been secured at Hyperion Hotel Hamburg. The organization will book your accommodation DIRECTLY to the Hotel, upon receipt of your deposit/ full payment.	
		Single accommodation E Double accommodation E	•		
Paym	ent	Payments should be made via paypal: conference@angelman.de Reference: ASA Conference / using your name			
Account Na	BAN /IFT	or to the following bank account: Sparkasse Neuss Angelman e. V. DE03 30550000 0093462885 WELADEDNXXX ASA Conference / your name We cannot accept credit or debit card payments.			
Cont	tact	If you have any questions or the weekend please co Mrs. Conny Schendle phone +49 179 217 58 9 e-Mail conny.schendler	ontact: r 8	ngs, payment 5/6	

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Registration Form

Please, fill, print, sign and return this registration form as soon as possible to:

Fax +49 2161 999 768 or E-Mail conference@angelman.de

Name of Organization / or private name (please mark):					
Participation: Thu / Fri / Sat (please mark)					
E-Mail Address (e.g. to inform you about changes):					
Name of Representatives:					
 Title, name #1					
Title, name #2					
Title, name #3					
Title, name #4					

Your registration will be completed with your full payment for the conference. Balance to be fully paid by **August 1, 2018**

Unless another participant is designated, cancellation within 14 days before conference (date of receipt of the cancellation notice) and no-shows will result in forfeiture of the entire participation fee. A EUR150 cancellation fee will be charged for cancellations prior to this date.

Cancellations and registrations must always be undertaken in writing.

The organizer reserves the right to change, postpone, relocate or cancel the entire event or parts thereof at short notice. Films and photos taken at the venue and possibly showing participants might be published for editorial purposes.