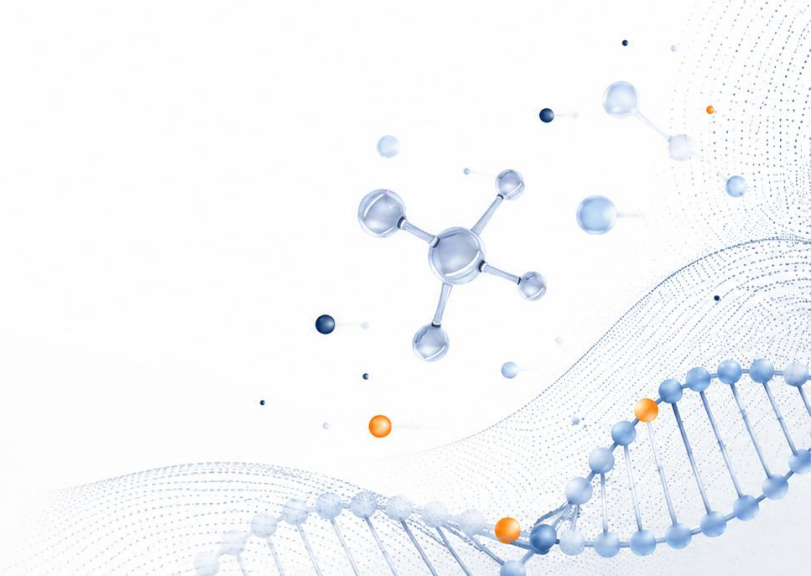




German Translational Oligonucleotide Symposium
— April 14-16 2027 —

Advancing oligonucleotide therapeutics from translational science to implementation.



Venue	Registration and Information	Contact
 Bürgerhaus Bahnstadt Gadamer Platz 1 69115 Heidelberg Germany	 Early registration open now! Visit our website for updates. www.gtos-symposium.de	info@gtos-symposium.de

DAY 1

Pre-symposium workshops & opening

Wednesday, 14 Apr

Workshops establish shared language across academia, industry, clinical translation and implementation.

12:00 – 14:00	Registration & welcome coffee Badge pick-up, informal arrival and orientation.
13:00 – 15:00	Pre-symposium workshops Industry sponsored, academic-initiated, tbc
15:00 – 15:30	Coffee Break Short break for informal networking.
15:30 – 17:15	Opening of GTOS 2027
15:30 – 15:45	Welcome and introduction to GTOS Rebecca Schüle and Holm Graessner (symposium presidents)
15:45 – 16:30	Opening keynote
17:15 – 19:30	Welcome reception, poster setup and networking <ul style="list-style-type: none">Informal networking with speakers, exhibitors and poster presenters,Poster setup,Exhibition opening





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DAY 2 Scientific & translational foundations Thursday, 15 Apr

From disease rationale to preclinical evidence and clinical strategy.

08:30 – 8:40

Welcome to Day 2
Introduction to the sessions of the day.

8:40 – 10:40

Session 1: Disease translation — indication and unmet-need oriented
Speakers: Steve Petrou, Richard S. Finkel
3–4 abstract-selected presenters



Steve Petrou, PhD
Co-Founder and President of Research & Development, Praxis Precision Medicines, USA



Richard S. Finkel, MD
Director, Center for Experimental Neurotherapeutics, St. Jude Children’s Research Hospital, Memphis, USA

10:40 – 11:10

Coffee Break

13:00 – 15:00

Session 2: Preclinical translation — strategy, method and problem oriented
Speakers: Haiyan Zhou, H  l  ne Tran
3–4 abstract-selected presenters



Haiyan Zhou, MD, PhD
University College London, London, UK



H  l  ne Tran, PhD
Neurology Therapeutic Area, Servier Research & Development, Paris-Saclay, France

13:10 – 14:10

Lunch Break

14:10 – 16:40

Session 3: Clinical translation — strategy, method and problem oriented
Speakers: Timothy W. Yu, Andreas Brunklaus, Neil A. Shneider
3–4 abstract-selected presenters



Timothy Yu, MD, PhD
Boston Children’s Hospital; Harvard Medical School; Broad Institute of MIT and Harvard, Boston/Cambridge, USA



Andreas Brunklaus, MD
Professor of Childhood Epilepsy and Developmental Neuroscience, University of Glasgow; Consultant Paediatric Neurologist, Royal Hospital for Children, Glasgow, UK



Neil A. Shneider, MD, PhD
Claire Tow Professor of Motor Neuron Disorders and Director, Eleanor and Lou Gehrig ALS Center, Columbia University Irving Medical Center, New York, USA

16:40 – 17:10

Coffee Break

17:10 – 17:50

Flash poster talks I
Selected abstracts presented in 3–5 minute format.

17:50 – 18:40

Curated industry showcase
Short, practical presentations focused on translational bottlenecks rather than product marketing.

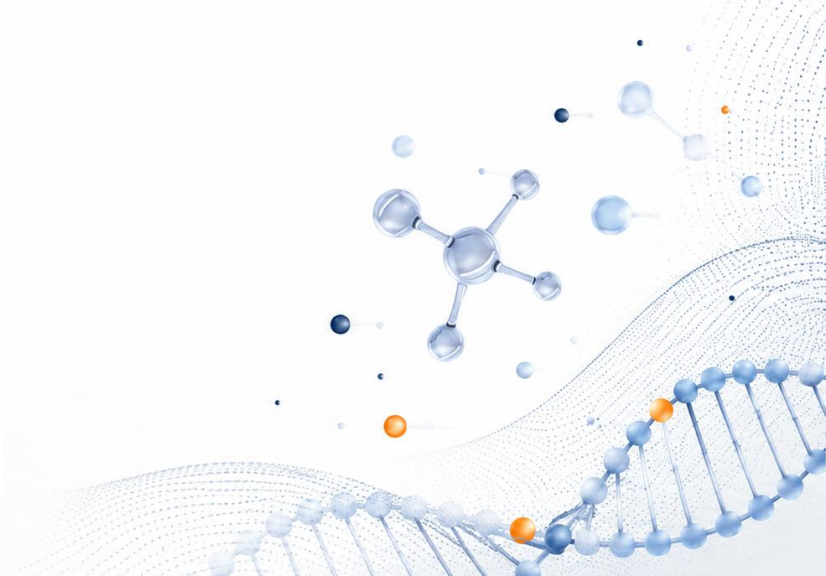
18:40 – 20:30

Poster session & networking reception
Integrated poster, sponsor and exhibitor area.



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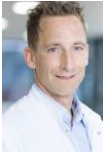


DAY 3

Implementation of ASO treatments

Friday, 16 Apr

Turning translational progress into responsible treatment pathways, evidence generation and sustainable infrastructure.

08:30 – 8:40	Welcome to Day 3 Introduction to the sessions of the day.
8:40 – 10:10	Session 4: Implementing ASO treatments for ultra-rare neurological diseases Speaker: Matthis Synofzik 3–4 abstract-selected presenters <div>Matthis Synofzik, MD Hertie Institute for Clinical Brain Research and Department of Neurodegenerative Diseases, University Hospital Tübingen; Gene and RNA Therapy Center Tübingen, Germany</div>
10:10 – 10:30	Coffee Break
10:30 – 12:15	Session 5: Regulatory and HTA development for individualized therapies in ultra-rare disease Speakers: Rebecca Ahrens-Nicklas, EMA representative – tbc 1–2 abstract-selected presenters <div>Rebecca Ahrens-Nicklas, MD, PhD Director, Gene Therapy for Inherited Metabolic Disorders Program; Associate Chief for Research, Division of Human Genetics, Children’s Hospital of Philadelphia; University of Pennsylvania, Philadelphia, USA</div>
12:15 – 13:00	Lunch Break
13:00 – 14:15	Session 6: Building translational infrastructure in Germany Safety monitoring, target engagement, biomarkers, trial design, endpoints, patient selection, follow-up and real-world evidence generation.
14:15 – 15:45	Session 7: Learning from other fields, other countries and other therapeutic modalities Speakers: <div><div><div>Tim Richardson</div><div>Richard S. Finkel</div><div>François Lamy</div><div>Thomas Opladen</div></div><div><i>CAR T Cell Therapy in Multiple Myeloma: Lessons in Clinical Translation, Toxicity Management, and Outpatient Delivery</i> <i>Gene-targeted precision therapies beyond oligonucleotides: translational lessons and new horizons from risdiplam</i> <i>Non-profit development of gene therapies: lessons for therapies in very small populations</i> <i>AADC gene therapy: lessons from implementing a highly specialized gene therapy</i></div></div>
	<div><div>Tim Richardson, MD Department I of Internal Medicine, Faculty of Medicine and University Hospital Cologne, University of Cologne, Germany</div><div>Richard S. Finkel, MD Director, Center for Experimental Neurotherapeutics, St. Jude Children’s Research Hospital, Memphis, USA</div><div>Jean-Francois Briand Operations and Scientific Innovation Director, AFM-Téléthon, France</div><div>Thomas Opladen, MD Section of Neuropediatrics and Metabolic Medicine, Heidelberg University Hospital, Germany</div></div>
15:45 – 16:05	Coffee Break
16:05 – 16:45	Flash poster talks II/Best abstracts High-impact abstracts from early-career investigators, selected poster highlights and award candidates.
16:45 – 17:30	Closing Session
16:45 – 17:00	Summary of scientific and implementation priorities emerging from GTOS 2027
17:00 – 17:05	Closing remarks