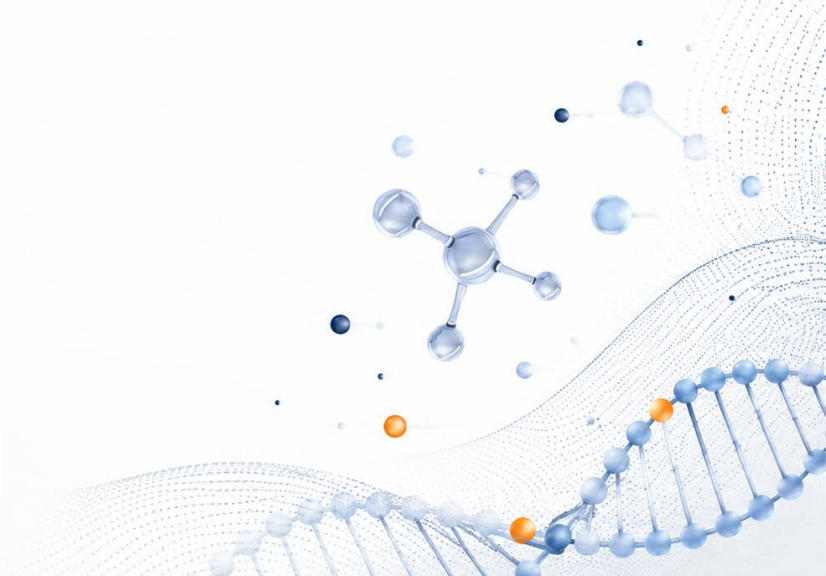




German Translational Oligonucleotide Symposium
— April 13-16 2027 —

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 scientific keynote Jonathan Watts, tbc <i>The translational state of the art in oligonucleotide therapeutics</i>
	
	Jonathan Watts, PhD — tbc RNA Therapeutics Institute, UMass Chan Medical School, Worcester, USA
16:30 – 17:15	Opening patient keynote Axel Lankenau <i>The role patient organizations in getting therapy ready</i>
	
	Axel Lankenau Chair, PCH-Familie e.V.; Co-founder, PCH2cure, Germany
17:15 – 19:30	Welcome reception, poster setup and networking - Informal networking with speakers, exhibitors and poster presenters, - Poster setup, - Exhibition opening



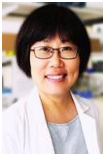


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DAY 2 Scientific & translational foundations Thursday, 15 Apr

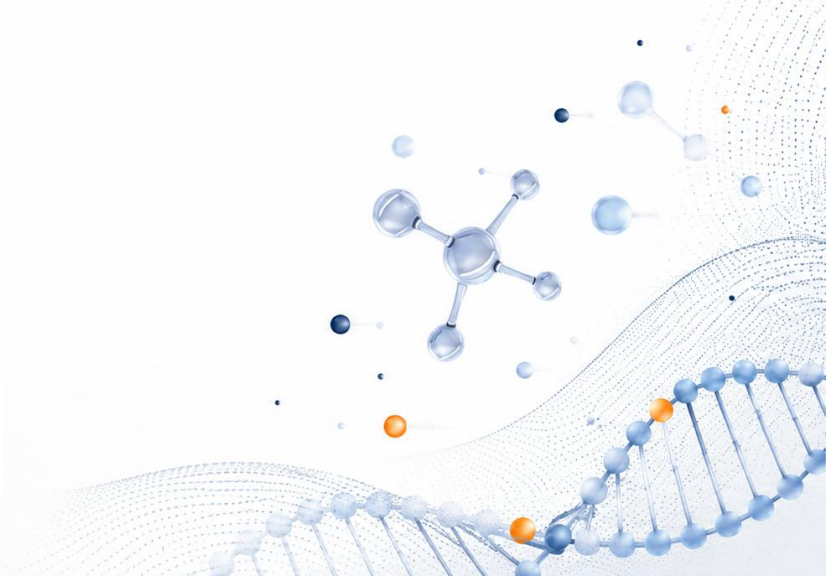
From disease rationale to preclinical evidence and clinical strategy.

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������ Tran 3–4 abstract-selected presenters
	 Haiyan Zhou, MD, PhD University College London, London, UK  H������ 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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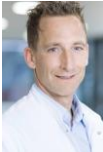


DAY 3

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 in the named-patient setting Speaker: Matthis Synofzik 3–4 abstract-selected presenters Matthis Synofzik, MD Hertie Institute for Clinical Brain Research and Department of Neurodegenerative Diseases, University Hospital Tübingen; Gene and RNA Therapy Center Tübingen, Germany
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 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
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: Tim Richardson Richard S. Finkel François Lamy Thomas Opladen <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> Tim Richardson, MD Department I of Internal Medicine, Faculty of Medicine and University Hospital Cologne, University of Cologne, Germany Richard S. Finkel, MD Director, Center for Experimental Neurotherapeutics, St. Jude Children’s Research Hospital, Memphis, USA Jean-Francois Briand Operations and Scientific Innovation Director, AFM-Téléthon, France Thomas Opladen, MD Section of Neuropediatrics and Metabolic Medicine, Heidelberg University Hospital, Germany
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