Joint scientific symposium of the TreatHSP network and the Tom Wahlig foundation

March 6th – 8th, 2024

Department of Molecular Neurology
Centre for Rare Diseases Erlangen (ZSEER)
Department of Stem Cell Biology

SOLD OUT
Wednesday, March 6th, 2024:
TreatHSP network meeting

Venue: Seminar room at TRC, Uniklinikum Erlangen, Kussmaulallee 10/12, 91054 Erlangen (parking garage directly connected)
https://goo.gl/maps/WiJJtWRGTBVLB3EGA

12.00 Registration, snacks
12.15 Internal meeting of the BMBF funded project PIs
12.45 Welcome note (R. Schüle, J. Winkler)
   Project updates and discussion of funded subprojects
   (10 min. report + 5 min. discussion)

Session 1 Chair: R. Schüle

13.00 Subproject 1: Coordination unit, central infrastructure and adaptive outcome validation
13.15 Subproject 2: Mobile gait Analysis to classify HSP-specific gait phenotypes and individualize symptomatic treatment of HSP
13.30 Subproject 3: Exploring the pathogenic and therapeutic impact of lipid homeostasis in SPG4 and SPG31 using iPSC-based neural models
13.45 Subproject 4: The role PI4K2A in autophagy and HSP

14.00 Coffee break

Session 2 Chair: B. Winner

14.15 Subproject 5: Development of an antisense oligonucleotide RNA therapeutic approach for POLR3A-associated HSP
14.30 Subproject 6: Linking dopaminergic degeneration and lysosomal dysfunction in SPG11/SPG15
14.45 Subproject 7: Therapeutic avenues for HSP caused by mutations in SPG7: targeting NAD metabolism and inflammation
15.00 Subproject 8: Neuroinflammation in SPG11: translational relevance and biomarkers

15.15 Coffee break

15.30 Interactive workshop to define a certified rater training of the mSPRS (M. Regensburger, R. Schüle, H. Gaßner)
16.30 Panel discussion to define consensus and needs for outcome measures in HSP

18.00 Dinner restaurant Steinbach Bräu,
   Vierzigmannstraße 4, 91054 Erlangen (walking distance 700 m)
   https://goo.gl/maps/UmVnU4JYUrUtaeg76
Thursday, March 7th, 2024:
Tom Wahlig symposium day 1

Venue: Uniklinikum Erlangen, Altes Universitätskrankenhaus, lecture hall Harald-zur-Hausen-Hörsaal, room 02.225, Krankenhausstr. 12, 91054 Erlangen https://goo.gl/maps/BSP9wVd1cJz7xyu8

Zoom-Participation: https://us06web.zoom.us/j/86147061601?pwd=wKEtnnvaw5RoSrvFodb0WS5ZLbjQDD.1
Meeting-ID: 861 4706 1601
Kenncode: 890493

09.00 Keynote lecture
John K. Fink (Ann Arbor, MI, USA)

09.45 – 12.00 Session 1:
Digital gait analysis in hereditary spastic paraplegias (HSP)
Chairs: H. Gaßner, L. Schöls

1. Multimodal movement analysis and future transfer into clinical care
   H. Gaßner (Erlangen, Germany)

2. Locomotor output and muscle activation patterns in HSP
   M. Serrao (Rome, Italy)

3. Progression of digital gait outcomes in trial-like timeframes for SPG4 and SPG7
   W. Ilg (Tübingen, Germany)

4. Rehabilitation interventions and their effect evaluation by instrumented analysis in people with HSP
   A. Geurts (Nijmegen, Netherlands)

11.30 – 13.00 Lunch break and poster session
(lecture hall: Hörsaal Östliche Stadtmauerstraße 11)

13.00 – 15.15 Session 2:
Intervention strategies and symptomatic therapy
Chairs: S. Klebe, M. Regensburger

1. German observational multicentre trial of the effects of botulinum toxin in HSP
   M. Regensburger (Erlangen, Germany)

2. Antispastic therapies in HSP: learnings from the SPASTOX and ZISPAST trials
   M. França (Campinas, Brazil)

3. Computational approach to precision neurostimulation
   A. Rowald (Erlangen, Germany)
15.15 – 15.30 Coffee break
(lecture hall: Hörsaal Östliche Stadtmauerstraße 11)

15.30 – 16.30 Session 3: Emerging disease mechanisms in HSP
Chairs: B. Winner, C. Depienne

1. Axon survival depends on ER-remolding via ER-phagy
   C. Hübner (Jena, Germany)

2. Microtubule defects in HSP
   A. Roll-Mecak (Bethesda, MD, USA)

18.00 Von Strümpell ceremony
Venue: Orangerie, “Wassersaal”, Wasserturmstraße 3, 91054 Erlangen
https://goo.gl/maps/wjwtwKMuVffQCw288

Chair: J. Winkler
Exhibition of Adolf von Strümpell
original scriptures and anatomical specimen
C. Wachter, University Archive Erlangen

Musical accompaniment
String quartet of the Friedrich-Alexander-Universität Erlangen-Nürnberg

140 years ago: medicine in the age of Strümpell
N. Metzger (Erlangen, Germany)

Historical view on HSP: HSP is coming home
M. Regensburger (Erlangen, Germany)

Spinal cord injury – another rare disease requiring multicentre efforts for successful clinical translation
N. Weidner (Heidelberg, Germany)

Reception with food and drinks

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Friday, March 8th, 2024:
Tom Wahlig Symposium Day 2

Venue: Lecture hall Harald-zur-Hausen-Hörsaal

Zoom-Participation: https://us06web.zoom.us/j/86147061601?pwd=wKEtnnvav5RoSrvFodb0WS5ZLbstQDD.1
Meeting-ID: 861 4706 1601
Kenncode: 890493

09.00 – 10.30 Session 4: Human cellular models
Chairs: B. Winner, D. Ebrahimi-Fakhari

1. Brain region-specific organoids as models of neurological disorders
   S. Mayer (Tübingen, Germany)
2. ER-mitochondria-signaling coupling in SPG7
   G. Casari (Naples, Italy)

3. Preclinical development of an AAV-based gene therapy for SPG5
   S. Hauser (Tübingen, Germany)

09.00 – 10.30  **Parallel workshop: movement analysis**
   Chair: V. Jakob, H. Gaßner
   Room: Motion lab (meeting point 9.00 a.m. at lecture hall)
   - Motion capture
   - EMG
   - Mobile gait analysis

10.30 – 10.45  **Coffee break**
   (Lecture hall: Hörsaal Östliche Stadtmauerstraße 11)

10.45 – 12:45  **Session 5: Disease-modifying therapies in HSP and beyond**
   Chairs: J. Winkler, L. Schöls

1. **N-of-1 antisense oligonucleotide trials for rare diseases**
   A. Aartsma-Rus (Leiden, Netherlands)

2. **Emerging therapies for adrenomyeloneuropathy**
   W. Köhler (Leipzig, Germany)

3. **SBT101 gene therapy for adrenomyeloneuropathy**
   F. Eichler (Boston, MA, USA)

12.45 – 14.00  **Lunch break and poster session**
   (Lecture Hall: Hörsaal Östliche Stadtmauerstraße 11)

14.00 – 15.15  **Award ceremony**
   - TWS prize awardee
   - Von Strümpell prize awardee
   - Poster prize awards

15:15 – 15:30  **Concluding remarks**
Sponsoring/Industrial booth

Amylyx Pharmaceuticals
1.500,– Euro

Ipsen Pharma GmbH
4.000,– Euro

Otto Bock HealthCare Deutschland GmbH
3.000,– Euro

Portabiles HealthCare Technologies
500,– Euro

Certification

CME-points are applied for.
Organization committee

Uniklinikum Erlangen
- PD Dr. Heiko Gaßner
- PD Dr. Martin Regensburger
- Prof. Dr. Beate Winner
- Prof. Dr. Jürgen Winkler

University Hospital Essen
- Prof. Dr. Christel Depienne
- Prof. Dr. Stephan Klebe

University Hospital Heidelberg
- Prof. Dr. Rebecca Schüle

University Hospital Tübingen
- Prof. Dr. Ludger Schöls

City map legend

1. **Harald-zur-Hausen-Hörsaal** (Lecture hall)
   Krankenhausstraße 12
   (Entrance = Altes Universitätskrankenhaus)
   Tom Wahlig Symposium

2. **TRC**
   Kussmaulallee 10/12
   TreatHSP Symposium

3. **Orangerie**
   Wasserturmstraße 3
   Von Strümpell ceremony

4. **Hörsaal Östliche Stadtmauerstraße 11**
   Alternative entrance to poster session
Easy to reach

**CityLinie**
The bus line “CityLinie” is free of charge and brings you to Uniklinikum Erlangen every 15 minutes: from the central bus station e. g. via the stops Uniklinikum/Maximiliansplatz and Krankenhausstraße.

**By train**
Hauptbahnhof Erlangen (reachable by ICE train) is located approximately 1,000 metres from the Uniklinikum Erlangen.

**By car**
From the A 73 exit “Erlangen-Nord”, follow the signs saying “Parkhaus Uni-Kliniken“. There is a limited number of short-term and single-day parking spaces near the clinics. Please use the multi-storey car park “Parkhaus Uni-Kliniken” at Kussmaulallee (vehicle access via Palmsanlage).

Long-term parking is located at the parking area west of the railway station.