

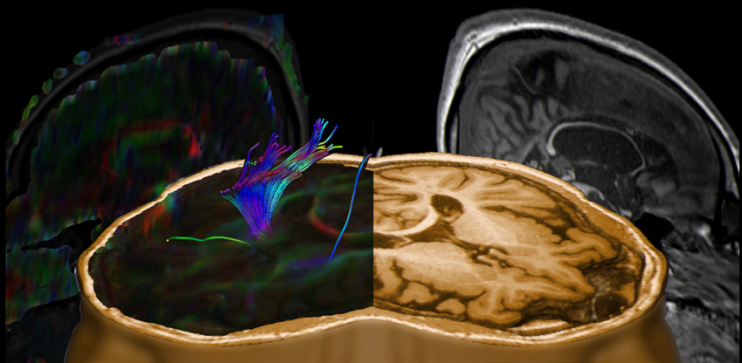
# Joint scientific symposium of the TreatHSP network and the Tom Wahlig foundation

**March 6<sup>th</sup> – 8<sup>th</sup>, 2024**

**Department of Molecular Neurology**

**Centre for Rare Diseases Erlangen (ZSEER)**

**Department of Stem Cell Biology**



**Uniklinikum  
Erlangen**



# Wednesday, March 6<sup>th</sup>, 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 7<sup>th</sup>, 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/BSP9wjVd1cJz7xyu8>

**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/wjwtkMuVffQCw288>

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**

## **Friday, March 8<sup>th</sup>, 2024:**

### **Tom Wahlig Symposium Day 2**

**Venue:** Lecture hall Harald-zur-Hausen-Hörsaal

**Zoom-Participation:** <https://us06web.zoom.us/j/86147061601?pwd=wKEtnnvaw5RoSrvFodb0WS5ZLbjQDD.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



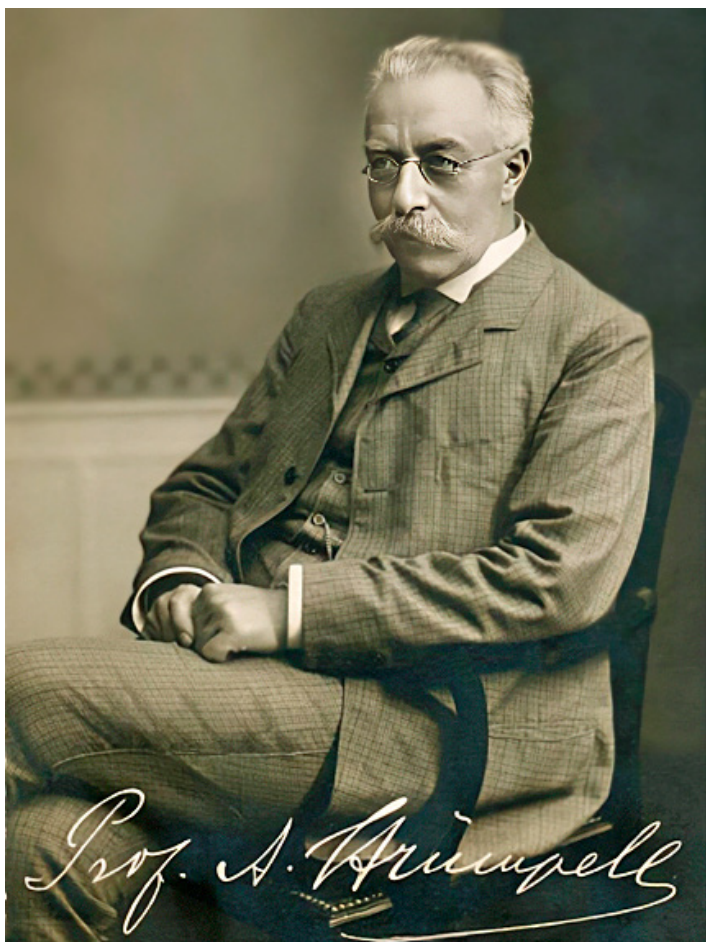
Otto Bock HealthCare Deutschland GmbH  
3.000,- Euro



Portables HealthCare Technologies  
500,- Euro

## Certification

CME-points are applied for.



# Cooperations

GEFÖRDERT VOM



Bundesministerium  
für Bildung  
und Forschung



**Interdisziplinäres**

**Zentrum für  
Klinische Forschung**



GRK 2162  
Neurodevelopment and CNS Vulnerability

*Forschungs-*  
**Stiftung**  
*Medizin*

**ZSEER**   
Zentrum für Seltene Erkrankungen Erlangen

# 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**.

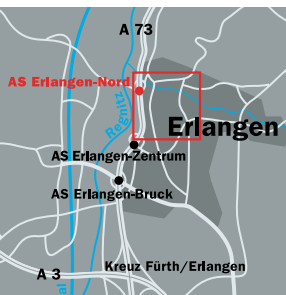
## By train

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.



## **Department of Molecular Neurology**

Head: Prof. Dr. med. Jürgen Winkler

Schwabachanlage 6 (Kopfkliniken), 91054 Erlangen

[www.molekulare-neurologie.uk-erlangen.de](http://www.molekulare-neurologie.uk-erlangen.de)

Tel.: 09131 85-39324

Fax: 09131 85-34672

[mn-sekretariat@uk-erlangen.de](mailto:mn-sekretariat@uk-erlangen.de)

Production of: Uniklinikum Erlangen/Kommunikation, 91012 Erlangen

Cover image: © Courtesy, Institute of Neuroradiology

Source: Adolf von Strümpell, Porträtsammlung der Universitätsbibliothek Erlangen-Nürnberg

[www.200.uk-erlangen.de/de/geschichte/20-koepfe-der-erlanger-universitaetsmedizin/struempell/index.html](http://www.200.uk-erlangen.de/de/geschichte/20-koepfe-der-erlanger-universitaetsmedizin/struempell/index.html)