BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors. Follow this format for each person. **DO NOT EXCEED FIVE PAGES.**

NAME: Weydt, Patrick

eRA COMMONS USER NAME (credential, e.g., agency login):

POSITION TITLE: Neurologist

EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable. Add/delete rows as necessary.)

INSTITUTION AND LOCATION	DEGREE (if applicable)	Completion Date MM/YYYY	FIELD OF STUDY
Freie Universität Berlin	MD	05/2000	Medicine (summa cum laude)
University of Washington, Seattle	Postdoctoral	09/2006	Neuroscience
Ulm University	Boards	05/2013	Neurology
Ulm University	Habilitation	02/2016	General Neurology
Bonn University	Faculty	02/2021	General Neurology

A. Personal Statement

I am a board certified neurologist at the University Hospital in Bonn, Germany, and have an affiliation as a clinician-scientist at the German Center for Neurodegenerative Diseases (DZNE) in Bonn.

At the University Hospital Bonn I run the Motoneuron disease clinic (ALS and SMA), which has expanded under my tenure from 30 to currently more than 250 patients per year. In addition, I head the Huntington's disease clinic, which I founded in 2016 and which sees around 100 patients per year. At the DZNE we run phase II and phase III trials (including ASO trials). My clinical and research efforts are driven by the conviction that providing excellent patient care today will attract patient populations to participate in translational innovative research programs that will enable the development of transformational treatment strategies tomorrow. My work has contributed to the establishment of informative and innovative biomarkers, such as neurofilaments, troponins and body weight. The regulatory implications have lead to me being selected as a permanent member of the Science Advisory Group (SAG) Neurology of the European Medicines Agency (EMA) in 2020.

I have a background in investigating metabolic and inflammatory aspects of neurodegeneration in general and ALS and Huntington disease in particular at the laboratory and clinical level. As PI or co-Investigator on several university- and industry-funded grants, work at the interface of clinical and basic research of two paradigmatic diseases. As a result of these previous experiences, I am aware of the importance of frequent communication among project members and of constructing a realistic research plan, timeline, and budget. Patient and community out-reach is very important to me and I organize the very successful "Bonner ALS Tag" series since 2018 and run social media channels on YouTube, X and Instagram (@als_doc, @huntington_doc)

Bibliographical indices (Google Scholar 1.6.2024): h-index: 45; Citations: 8370

Recent relevant publications:

- Meyer T, Schumann P, Weydt P, Petri S, Koc Y, Spittel S, Bernsen S, Günther R, Weishaupt JH, Dreger M, Kolzarek F, Kettemann D, Norden J, Boentert M, Vidovic M, Meisel C, Münch C, Maier A, Körtvélyessy P. Neurofilament light-chain response during therapy with antisense oligonucleotide tofersen in SOD1-related ALS: Treatment experience in clinical practice. Muscle Nerve. 2023 Jun;67(6):515-521. doi: 10.1002/mus.27818. Epub 2023 Apr 3. PMID: 36928619
- 2. Kittipeerapat N, Fabian R, Bernsen S, **Weydt P***, Castro-Gomez S. Creatine Kinase MB Isoenzyme Is a Complementary Biomarker in Amyotrophic Lateral Sclerosis. Int J Mol Sci. 2023 Jul 20;24(14):1168
- 3. Dorst J, **Weydt P****, Brenner D, Witzel S, Kandler K, Huss A, Herrmann C, Wiesenfarth M, Knehr A, Günther K, Müller K, Weishaupt JH, Prudlo J, Forsberg K, Andersen PM, Rosenbohm A, Schuster J, Roselli F, Dupuis L, Mayer B, Tumani H, Kassubek J, Ludolph AC. Metabolic alterations precede neurofilament changes in presymptomatic ALS gene carriers. EBioMedicine. 2023 Apr;90:104521.
- Lipsmeier F, Simillion C, Bamdadian A, Tortelli R, Byrne LM, Zhang YP, Wolf, D, Smith AV, Czech C, Gossens C, Weydt P, Schobel SA, Rodrigues FB, Wild EJ, Lindemann M. A Remote Digital Monitoring Platform to Assess Cognitive and Motor Symptoms in Huntington Disease: Cross-sectional Validation Study. J Med Internet Res. 2022 Jun 28;24(6):e32997.
- 5. Castro-Gomez S, Radermacher B, Tacik P, Mirandola SR, Heneka MT, **Weydt P**. Teaching an old dog new tricks: serum troponin T as a biomarker in amyotrophic lateral sclerosis. Brain Commun. 2021 Nov 17;3(4)
- 6. Meyer T, Schumann P, Weydt P, Petri S, Koc Y, Spittel S, Bernsen S, Günther R, Weishaupt JH, Dreger M, Kolzarek F, Kettemann D, Norden J, Boentert M, Vidovic M, Meisel C, Münch C, Maier A, Körtvélyessy P. Neurofilament light-chain response during therapy with antisense oligonucleotide tofersen in SOD1-related ALS: Treatment experience in clinical practice. Muscle Nerve. 2023 Jun;67(6):515-521. doi: 10.1002/mus.27818. Epub 2023 Apr 3. PMID: 36928619

B. Positions, Scientific Appointments, and Honors

Positions and Scientific Appointments

10/2016-present	Consultant Neurologist, Research Group Leader, Department of	
	Neurodegenerative Diseases, Bonn University/DZNE	
2016	Habilitation, Clinical Neurology, Ulm University (Umhabilitation to	
	Bonn 2021)	
2013-2016	Board certified Neurologist, Department of Neurology, Ulm University	
2006-2013	Residency Department of Neurology, Ulm University	
2009-2010	Residency Department of Psychiatry, Ulm University	
2003-2006	Senior Fellow, Dept. Lab Medicine, University of Washington	
2000-2003	Senior Fellow, Dept. Neurology, University of Washington	
1997-2000	Residency Department of Neurology, LMU Munich	

^{*} shared last authorship, ** shared first authorship

Honors (selection)				
2023	Poster Prize ENCALS meeting Barcelona			
2022	3 rd Prize, Pflegepreis der DGN (Patient Care Award, German Society of			
	Neurology)			
2021-ongoing	Member of the Scientific Advisory Group (SAG) of the European Medicines			
	Agency (EMA) (2021-)			
2018-ongoing	Co-Chair of the Executive Committee (EC) of the European Huntington Disease			
	Network (EHDN); Chair of the Program Committee of the EHDN Plenary			
	Meeting 2020-2023, including two virtual meetings			
2017	Poster Prize ENCALS meeting Tours, France			
2020-onging	Founder and Speaker of the B-Centers for Motoneuron Diseases and for			
	Choreatic Movement Disorders at the Center for Rare Diseases Bonn (ZSEB)			
2016	Elected Member of the Executive Committee (EC) of the European Huntington			
	Disease Network (EHDN)			
2012-16	Elected member of the Scientific and Bioethical Advisory Board (SBAC) of the			
2212	European Huntington Disease Network (EHDN)			
2012	Seed Grant EHDN: Smartphone App and Kinect as tools to assess motor			
2011	function in Huntington Disease – a Pilot Study (12,500 €)			
2011	Head of the Organizing Committee of the FENS/IBRO Summer School on			
2000	"Metabolic Aspects of Chronic Brain Disease" Reisensburg (ca. 32,000 €)			
2009	Pilot Grant Thierry Latran Foundation "The role of PGC-1 in ALS" (100,000 €)			
2009	Poster Prize Neurogenetics, Deutsche Gesellschaft für Neurologie			
2009	Head of the Organizing Committee of the PENS Summer School on "Metabolic			
2005	Aspects of Chronic Brain Disease" Reisensburg (40,000 €)			
2005	Platform Presentation "PGC-1\(\) transcription interference produces deranged			
	thermoregulation in Huntington's disease transgenic mice", ASHG meeting, Salt Lake City, UT, USA			
2005	Travel Stipend (\$US 1.500), Route28 Summit "Restoring Mobility: Stem Cells			
2003	and the Spinal Cord", Blaine, WA, USA			
2005	"Career Development Award", Muscular Dystrophy Association (MDA)			
2003	(\$US150.000) "VEGF Gene-therapy Strategies for Motor Neuron Disease"			
	(MDA3981) (award abandoned 2006 to return to Germany)			
2005	"Young Investigator Travel Award" (US\$1.200) and oral presentation, Gordon			
2005	Research Conference on CAG Repeat Disease, Mount Holyoke			
2000	Fellowship of the "Deutschen Akademie der Naturforscher Leopoldina" (Halle,			
	Saale) (ca. €50,000), "Die Rolle der Mikroglia in ALS"			

Complete List of Published Work in MyBibliography:

https://pubmed.ncbi.nlm.nih.gov/?term=weydt+p&sort=date

Clinical trial experience

	Official trial experience
1998-2000	Phase 3 Study: SR-57746 in Amyotrophic Lateral Sclerosis
	EudraCT No. 2005-002570-30, Investigator
2008-09	Phase 3 Study:ACR-16 in Huntington Disease,
	EudraCT No. 2009-0112334-99, Investigator
2007-2014	Observational study REGISTRY (EHDN), Investigator
2010	Observational study PREDICT, Investigator
2014-ongoing	Observational study ENROLL, Investigator
2014-2016	Phase 1/2 Study: BVS857 in Patients with SBMA, Investigator
2015	Phase 3 Study: LEGATO (Laquinimod in Huntington Disease), Investigator,
	EudraCT No. 2014-000418-75
2015-2017	Phase 1b Study: IONIS-HTTRx in with patients with Early Manifest HD,
	NCT02519036, local Co-PI (Ulm)
2019-2021	Phase 3 Study: GENERATION HD, NCT03761849 Local PI (Bonn)
2020-2023	Phase 2 Study: ROCK-ALS, NCT03792490 Local PI (Bonn)
2021-2023	Phase 3 Study: MT-1186, NCT04569084 Local PI (Bonn)
2022-2023	Phase 3 Study: COURAGE-ALS, NCT04944784 Local PI (Bonn)
2022-ongoing	Phase 2 Study: DAZALS, NCT05407324 Local PI (Bonn)
2023-ongoing	Phase 1 Study: QRL-201, NCT05633459 Local PI (Bonn)
2023-ongoing	Phase 2 Studs: GENERATION HD2, NCT05686551 Local PI (Bonn)

PD Dr. Patrick Weydt

Bonn June 12th 2024