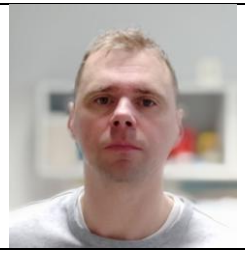
PROFILE FOR INSTITUTE WEBSITE

Current photo in graphic file e.g. 2024, 2023.	
Name and surname, Title	**Pawel Lisowski, PhD, DSc, Eng.**
Position	Adiunkt
Hirsch Index and Number of citations (according to Scopus) on the day of completing the form	H index 23, Total number of citations: 2082
Research areas (in points, min. 200 characters, max. 500 characters)	– Gene therapy technology development; Genome engineering; Gene editing; Somatic cell gene editing; Designer nucleases; Base editors; Prime editors; Homology independent sequence replacement. – Gene editing toolbox delivery into the brain; Viral Like Particles (VLPs); Lipid Nanoparticles (LNs); recombined AAVs. – Stem cell engineering; Cell fate reprograming; Stem cell reprograming; Stem cell-based disease modeling; – Somatic cell engineering; Neurotechnology; Post-mitotic neuron engineering; Brain organoids engineering; Guided organoids; Unguided organoids. – Neurodevelopmental disease; Undiagnosed disease; Rare disease; Neuropsychiatric disease. – IRF2BPL-related disorders; polyQ-related neurodevelopmental disorders; Leigh Syndrome; SynGAP1 Syndrome; NEDAMSS. – Clinical WES/WGS support “beyond exome” diagnostics. – Functional genomics; Single cell RNA-seq (scRNAseq), Spatial transcriptomics. – Human 3D model system High Throughput Screening (HTS); drug repurposing.
Total number of completed research projects; currently implemented research projects (title and number) and selected max. 3 completed projects (title and number) from the newest ones, i.e. 2024, 2023, 2022…	Total number of completed research projects: 10 currently implemented research projects (title and number) – FOCUS (From mOleCUlar origins to treatment of IRF2BPL-related disorderS). Alliance4Rare 2023-2026 Eva Luise und Hosrt Köhler Stiftung Forschungsprojekt förderung Klinik für Kinder- und Jugendmedizin – Gene Therapy of Rare Diseases (GetRadi). 2023-2027 EU-funded Marie Skłodowska-Curie Project.

	– CureMILS. A reprogramming-based strategy for drug repositioning in patients with mitochondrial DNA-associated Leigh Syndrome. 2021-2025 Project funded by the European Joint Programme on Rare Disease. – Functional study of the nonsense mutation in the IRF2BPL gene – an attempt to characterize a novel neurodegenerative human disease. 2018–2024 National Science Center OPUS Grant No. 2017/27/B/NZ1/02401 – selected max. 3 completed projects (title and number) from the newest ones – Harnessing the mitochondrial vulnerability of neurons from Huntington's Disease patients for uncovering novel therapeutic targets. 2020-2024 Deutsche Foschunsgemeinschaft (DFG) project #PR1527/5-1 – Decoding genetic predisposition to rare neurodevelopmental disorders using genome engineering and patient-specific pluripotent stem cell based neuronal modeling. – 2017-2022 National Science Center Grant No. 2016/22/M/NZ2/00548. – Development of humanized neurodegenerative diseases models using TAL-Effector Nucleases (TALENs). DPN/MOB109/II/2012; 2013-2015 (Project Leader P. Lisowski).
Total number of publications; ORCID (number and hyperlink to the profile); SCOPUS (number and hyperlink to the profile); indicate selected publications (max. 5)	Total number of publications: 46, Scopus Author Identifier: 15127146700 SCOPUS hyperlink: https://www.scopus.com/authid/detail.uri?authorId=15127146700 selected publications (max. 5) Mutant Huntingtin impairs neurodevelopment in human brain organoids through CHCHD2-mediated neurometabolic failure. Lisowski P, Lickfett S, Rybak-Wolf A, Pentimalli TM, Jüttner R, Glažar P, Uppal K, Bottani E, Brunetti D, Secker C, Zink A, Meierhofer D, Henke MT, Dey M, Ciptasari U, Mlody B, Hahn T, Berruezo-Llacuna M, Karaiskos N, Di Virgilio M, Mayr JA, Wortmann SB, Gotthardt M, Jones DP, Mayatepek E, Stenzel W, Diecke S, Kühn R, Wanker EE, Rajewsky N, Schuelke M, Priller J, Prigione A. Nat Commun. 2024. Mechanisms of IRF2BPL-related disorders and identification of a potential therapeutic strategy. Sinha Ray S, Dutta D, Dennys C, Powers S, Roussel F, Lisowski P, Glažar P, Zhang X, Biswas P, Caporale JR, Rajewsky N, Bickle M, Wein N, Bellen HJ, Likhite S, Marcogliese PC, Meyer KC. Cell Rep. 2022 Dec 6;41(10):111751. doi: 10.1016/j.celrep.2022.111751.

	Defective metabolic programming impairs early neuronal morphogenesis in neural cultures and an organoid model of Leigh syndrome. Inak G, Rybak-Wolf A, Lisowski P, Pentimalli TM, Jüttner R, Glažar P, Uppal K, Bottani E, Brunetti D, Secker C, Zink A, Meierhofer D, Henke MT, Dey M, Ciptasari U, Mlody B, Hahn T, Berruezo-Llacuna M, Karaiskos N, Di Virgilio M, Mayr JA, Wortmann SB, Priller J, Gotthardt M, Jones DP, Mayatepek E, Stenzel W, Diecke S, Kühn R, Wanker EE, Rajewsky N, Schuelke M, Prigione A. Nat Commun. 2021 Mar 26;12(1):1929. doi: 10.1038/s41467-021-22117-z. Neurodevelopmental disorder associated with IRF2BPL gene mutation: Expanding the phenotype. Skorvanek M, Dusek P, Rydzanicz M, Walczak A, Kosinska J, Kostrzewa G, Brzozowska M, Han V, Dosekova P, Gdovinova Z, Lehotska Z, Lisowski P*, Ploski R. Parkinsonism Relat Disord. 2019 May:62:239-241. doi: 10.1016/j.parkreldis.2019.01.017. Epub 2019 Jan 24. Mitochondria and the dynamic control of stem cell homeostasis. Lisowski P, Kannan P, Mlody B, Prigione A. EMBO Rep. 2018 May;19(5):e45432. doi: 10.15252/embr.201745432. Epub 2018 Apr 16. *Corresponding author
Total number of patents; selected patents (max. 2) and a hyperlink to personal patent achievements (UP RP), on the day of completing the form	– 1 US patent pending: Homology independent sequence replacement in human post mitotic neuron.
Selected scientific achievements from the newest, i.e. 2023, 2022, 2021... (in points, min. 800 characters, max. 1000 characters)	– Patient specific induced pluripotent stem cell (iPSC)-driven drug discovery approach enabled the identification of sildenafil as a drug repurposed for the treatment of children affected by Leigh syndrome (Nature Medicine, 2024). – In frame deletion of the polyQ sequence in human stem cell and post mitotic neuron of HTT gene as a gene therapy strategy in Huntington Disease (Nature Communication 2024). – Development of in vitro model of gene therapy for SynGAP1 syndrome using base editing in children carrying stop variants (American Society of Gene & Cell Therapy, 2024). – Discovery of new polyQ related neurodegenerative and neurodevelopmental disorder related to mutations in IRF2BPL gene leading to lately named NEDAMSS syndrome (Parkinsonism Relat Disord 2019) – Development of in vivo and vitro models of a gene therapy for NEDAMSS syndrome using base editing in children carrying IRF2BPL stop variants (American Society of Gene & Cell Therapy, 2024).

	– Discovery of neurometabolic failure through CHCHD2 dysregulation in patients with Huntington's Disease and potential treatment strategy (Nature Communication 2024). – Identification of IRF2BPL-related mechanism in children with nonsense variants of IRF2BPL gene (Cell Reports 2023).
Number and list of defended PhD students from the latest, i.e. 2024, 2023, 2022...	– 1 defended, 4 ongoing: – Daniel Bauersachs: Establishing gene therapy tools for neurodevelopmental disorders using a next generation base editor. – Louise Bomholtz: Establishing gene therapy tools for IRF2BPL-related neurodevelopmental disorder using base- and prime editing. – Sara del Rey: High content drug repurposing for IRF2BPL related disorders using micropatterned brain organoid system. – Ludovica Rigat: Investigating the role of microglia in neurodegeneration using 3D human brain model system.
Organizational activities, dissemination of knowledge and others (in points, min. 300 characters, max. 1000 characters)	Afiliated with: – Klinik für Psychiatrie, Charité – Universitätsmedizin Berlin, Germany; – Berlin Institute for Medical Systems Biology (BIMSB), Berlin, Germany; – Max-Delbrück-Center for Molecular Medicine (MDC) in the Helmholtz Association, Berlin, Germany; – Universitätsklinikum Düsseldorf (UKD), Germany. Other appointments: Leader of US-EU IRF2BPL research network under Research Collaboration Agreements (RCAs) with: - Within United States: Center for Gene Therapy, Nationwide Children's Hospital, Columbus, Ohio; Mayo Clinic Jacksonville, FL; University of California San Diego (UCSD), CA; Salk Institute, La Jolla, CA; Sanford Health, Sieux Falls (ND); Nemours Children's Hospital Wilmington (DE). - Within EU: Helmholtz Zentrum München; University Medical Center Utrecht; Universitätsmedizin Göttingen; MSH Medical School Hamburg; Københavns Universitet. Co-founder of IRF2BPL.DE research initiative (IRF2BPL Support Group Europe e.V.) aimed in funding and propagation of research and treatment of new neurodevelopmental and neurodegenerative IRF2BPL-related disease called NEDAMSS syndrome: https://irf2bpl.de/ More information about Dr. Lisowski research can be found here: https://www.functionalgenomics.pl/