The IIIrd International Scientific Conference on the occasion of World Duchenne Awareness Day 2025
Kozminski University

"Only Together Can We Defeat this Disease"

"Translation Medicine in DMD/BMD and Other Neuromuscular Diseases"

Conference Moderator: Lukasz Wiech, MD

Hour		Agenda Item	Topic	Speaker/Panelist	
from	to			Full Name	Institution / Affiliation
Day 1					
Part I – Basic Research					
8:00	9:00	Registration	Participant Registration		
9:00	9:10	Welcome	Guest Welcome	Dr Łukasz Więch, MD	Kozminski University Warsaw
				Prof. dr hab. n. med. Anna Kostera-Pruszczyk	Warsaw Medical University Warsaw
				Prof. Józef Dulak	Jagiellonian University Krakow
					StopDuchenne Foundation Warsaw
9:10	10:10	Opening Lecture	Development of next generation gene therapies for Duchenne muscular dystrophy	Prof. Jeffrey S. Chamberlain.	University of Washington USA
10:10	10:40	Lecture	Novel Strategies to Improve the Therapeutic Efficiency of mRNA	Prof. Jacek Jemielity	Warsaw University
10:40	11:10	Lecture	Stem cells in modeling disease mechanisms and testing potential treatments	Prof. Józef Dulak	Jagiellonian University Krakow
11:10	11:50	Break	Coffee and Informal Talks		
11:50	12:20	Lecture	Ex vivo gene therapy for Duchenne Muscular dystrophy	Prof. Giulio Cossu	The University of Manchester UK
12:20	12:50	Lecture	From understanding LAMA2-related muscular dystrophy to innovative dual AAV gene therapy	Prof. Markus Rüegg	University of Basel Switzerland
12:50	13:20	Lecture	Hydrogen sulfide-based therapeutics in Duchenne muscular dystrophy: a preclinical evaluation in dystrophic mice	Prof. Agnieszka Łoboda	Jagiellonian University Krakow
13:20	13:50	Lecture	Approaches for gene therapy of dominant muscular dystrophies	Prof. Joel Chamberlain	University of Washington USA
13:50	14:50	Break	Lunch and Informal Talks		
14:50	15:20	Lecture	P2X7 receptor as a therapeutic target in DMD - mechanisms and opportunities	Prof. Dariusz C. Górecki MD. PhD	School of Medicine, Pharmacy and Biomedical Sciences; University of Portsmouth, UK
Day 1					
Part II – Clinical Research and Clinical Applications					
15:20	15:35	Lecture	P2X7 receptor in non-commercial clinical trials: "To Slow DMD" – Project financed by the Medical Research Agency (ABM)	Dr hab. n. med. Anna Potulska-Chromik PhD Prof. dr hab. n. med. Anna Kostera-Pruszczyk	Warsaw Medical University
15:35	16:05	On-Line Lecture	Explain the clinical exon skipping therapy development I was involved in, highlighting how things failed because of bad preparation and making wrong assumptions (and of course what we can learn from this)	Prof. Annemieke Aartsma-Rus	Leiden University The Netherlands
16:05	16:40	Break	Coffee and Informal Talks		
16:40	18:10	Panel Discussion	"New Therapies in Basic Research – From Concept to Promising Results" Panel discussion with leading researchers on how laboratory discoveries are translated into potential therapies. The conversation will focus on promising treatment approaches for Duchenne muscular dystrophy (DMD) and other neuromuscular diseases (such as LAMA2-CMD, myotonic dystrophies, LGMD, etc.) that are in early stages of development.	Moderator Prof. Józef Dulak Prof. Jeffrey S. Chamberlain, Prof. Joel Chamberlain Prof. Joel Chamberlain Prof. Giulio Cossu, Prof. Markus Rüegg, Dr Jean-François Briand PhD Prof. Agnieszka Łoboda Prof. Darek Górecki Prof. Anna Kostera-Pruszczyk Prof. Annemieke Aartsma-Rus	
18:10	18:20	Photo	Group Photo of the Conference Participants		
18:20		**End of Day One of the Conference**			

Hour		Agenda Item	Topic	Speaker/Panelist	
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Day 2					
Part III – Clinical Research and Clinical Applications					
9:00	9:30	Lecture	From Parexel's perspective as a leading CRO • Review of current key developments in DMD clinical trials • Making clinical trials more patients friendly • Involving patients early on pharma / biotech drug developments to provide input into programs and project level documents • Partnering between CRO and patient advocacy organizations: opportunities and challenges	Rachel Smith (Rare Disease Therapeutic Area Lead) Dominika Wolska-Mularzuk (Project Leader)	Parexel international
9:30	10:00	Lecture	TBC (Title to be confirmed)	Dr Jean-François Briand PhD	AFM Telethon - France
10:00	10:30	Lecture	To Slow DMD – A Phase III Non-Commercial Clinical Trial Aimed at Slowing the Progression of Duchenne Muscular Dystrophy (DMD) – Project Funded by the Medical Research Agency (ABM)	Dr hab. n. med. Anna Potulska-Chromik PhD Prof. dr hab. n. med. Anna Kostera-Pruszczyk	Warsaw Medical University
10:30	11:00	Lecture	"DMD treatment available in Europe and worldwide"	Dr hab. n. med. Anna Potulska-Chromik PhD	Warsaw Medical University
11:00	11:10	Industry presentation	Presentation of innovative assistive devices and mobility solutions for people with disabilities, offered by Wcheelchair Centre Lublin		Wcheelchair Centre Lublin
11:10	11:50	Break	Coffee and Informal Talks		
11:50	12:20	Lecture	Improving and Optimizing Clinical Trials – The Importance of the Patient Perspective in Their Design and Implementation	Dr Hanna Preus MBA, FRQA, MICR	TFS HealthScience Imperial College Business School London UK
12:20	12:40	Lecture	Safety First – What Every Patient Should Know About Clinical Trials and New Medicines	Dr n. o zdr. Weronika Lepionka	Expert, Team Leader, Senior Safety Drug Manager - Pfizer
12:40	13:00	Lecture	Legal Safety of Patients in Clinical Trials: Consent, Compensation, Liability	Piotr Zięcik, Attorney-at-law	Attorney-at-Law, Partner at Zięcik, Miłowska i Partnerzy
13:00	13:30	Lecture	Drug Reimbursement Mechanisms in Rare Diseases and the Role of Patients	Izabela Pieniazek, MSc	Senior Director Centre of Excellence at Global Evidence Synthesis Department - Arcana a Certara Company
13:30	13:45	Lecture	Validation of α-synuclein Modifications in Parkinson's dIsoRder Evolution (VaMpiRE)	Dr hab. Tomasz Stępień PhD	IPIN Warszawa
13:45	13:55	Industry presentation	Innovative Transfer Devices in Daily Care – Patient Safety and Caregiver Comfort		Winncare Polska Sp. z o.o.
13:55	14:55	Break	Lunch and Informal Talks		
14:55	15:15	Lecture	Introduction to Panel Discussion. How Parents Became Research Initiators A patient advocate representing individuals affected by ultra-rare diseases will share a personal story – from the diagnosis of his daughter's ultra-rare genetic condition to the founding of an international research foundation. This introduction will highlight the story of PACS2, the challenges and achievements of grassroots efforts toward therapy development, and will set the stage for the following panel discussion.	Piotr Kośla	PACS2 Research Foundation
15:15	16:45	Panel Discussion	"From Research to Therapeutic Applications – How to Accelerate and Unblock the Path to New Medicines" This panel will bring together clinicians, scientists, representatives of pharmaceutical companies, and patient advocates to discuss how promising research results can be effectively translated into accessible therapies. The discussion will explore existing regulatory, financial, and organizational barriers — and consider which solutions can be implemented quickly and which require long-term investment and structural change. The goal is to envision how Poland can become an innovative, dynamic hub for scientific and clinical research: a place where pharmaceutical companies want to invest and where patients gain faster access to cutting-edge treatments.	Moderator: Piotr Sawicki - Luxmed Onkologia Panelists: Dr Jean-François Briand - AFM Thelethon Ireneusz Staroń - Z-ca Prezesa ABM Prof. Anna Kostera-Pruszczyk - WUM Dr Aldona Chmielewska - AGO Alliance Poland Foundation Magdalena Margasińska-Brach - Caidya Kinga Adamczyk	
16:45	16:50	Closing Remarks	Summary and Official Conclusion Representatives of the organizing committee will summarize the key points of the discussions and officially close the conference. They will thank the speakers, participants, and sponsors for their valuable contributions. The closing remarks will emphasize our shared mission and the importance of continuing our collective efforts — because only together can we overcome this disease.		
16:50	17:00	Photo	Group Photo of the Conference Participants		
17:00		**Conference Closing**			