



For the promotion
and recognition
of excellence
in orphan diseases
treatment research

The Review Committee

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THE WINNER OF THE 11TH EDITION OF THE INTERNATIONAL PRIZE FOR SCIENTIFIC RESEARCH ARRIGO RECORDATI ANNOUNCED

The 2024 Award was dedicated to the promotion and recognition of excellence in research within paediatric oncology, specifically neuroblastoma

The project from **Dr. Adam Durbin, M.D., PhD**, which aims to provide new potential therapeutic strategies for children with neuroblastoma, was awarded with a **€100,000 research grant**

Milan, May 16th, 2024

At the 5th Annual Meeting of the European Society for Paediatric Oncology (SIOP) held in Milan yesterday, the 11th edition of the International Prize for Scientific Research Arrigo Recordati was awarded to Dr. Adam Durbin¹ from St. Jude Children's Research Hospital in Memphis for providing new potential therapeutic strategies for children with neuroblastoma.

The Prize was established in 2000 by Recordati Group in memory of the Italian pharmaceutical entrepreneur Arrigo Recordati to perpetuate his legacy and to inspire biomedical discoveries benefiting people worldwide. Arrigo Recordati strongly believed in the power of research to drive the development of the pharmaceutical industry and provide products beneficial to public health and individual well-being.

The Prize received more than 40 applications from junior researchers of different nationalities, which were assessed by the 2024 Review Committee - an independent panel of internationally recognized experts who have provided leadership throughout their long careers in the field of rare diseases, including Chairman Prof. Robert J. Desnick M.D., PhD., D.SC., Prof. Andrew Pearson, M.D., PhD., and Prof. Charles Patrick Reynolds, M.D., PhD.

During the Award ceremony, the Review Committee and Andrea Recordati, Chairman of Recordati, were delighted to announce that **Dr. Adam Durbin's project was awarded with the € 100,000 Arrigo Recordati Prize.**

*"I am proud we have given an important contribution to further support research within paediatric oncology, specifically neuroblastoma, which is a rare devastating condition for the children diagnosed with it and for their families – it reflects our strong commitment to help the few who suffer from rare diseases through our dedicated business unit Recordati Rare Diseases," said **Andrea Recordati, Chairman of Recordati**, announcing the winner. "It is an honour and a privilege to award the prize to Dr. Adam Durbin's outstanding research project, which will offer the opportunity to provide new potential therapeutic strategies for children with neuroblastoma".*

¹ Dr Adam Durbin, M.D.,PhD., is Assistant Member at the Department of Oncology at St. Jude Children's Research Hospital in Memphis, Tennessee, USA

The winning project: “Targeting E/P300/CBP Control of Chemoresistance in Neuroblastoma”

A major challenge in cancer medicine is the management of relapsed disease. Relapse has classically been associated with acquired mutations that confer therapeutic resistance. However, emerging evidence indicates that cancer cells may shift between epigenetically specified transcriptional cell states, some of which may be resistant to chemotherapy. Relapsed cancer and resistance to chemotherapy are major drivers of poor patient outcomes in the relatively mutationally-quiet high-risk neuroblastoma. Neuroblastoma exists in distinct cell states, termed “adrenergic” (ADRN) and more chemo-resistant state, termed “mesenchymal” (MES). The MES cell state is rare in cell culture, patient-derived xenografts (PDXs) and human tumours, but relatively enriched at patient relapse. Further, these states may interconvert under genetic or pharmacological stresses, supporting a hypothesis that MES cells may form a reservoir for repopulation of ADRN-dominant tumours after conventional therapies. How these cell state switches occur, and how they can be controlled, remain unknown.

Thus, using new reporter systems in combination with state-of-the-art CRISPR-cas9-based knocking technologies and pharmacological inhibitor and degrader compounds, we will interrogate regulators of enhancer biology to identify controllers of chemoresistance. These experiments will identify the mechanisms by which enhancer perturbation controls cell state and chemoresistance and determine optimal pharmacologic strategies to enhance chemosensitivity in new high-fidelity murine models of neuroblastoma. These studies are thereby poised to provide new potential therapeutic strategies for children with neuroblastoma.

2024 Award Winner: Dr. Adam Durbin

Adam David Durbin, M.D., PhD., received his B.Sc (Hons) in Biology from York University, followed by his Medical degree and PhD at the University of Toronto. His PhD was performed in the Department of Medical Biophysics, University of Toronto, under the supervision of Dr. David Malkin, studying signal transduction mechanisms in paediatric rhabdomyosarcoma.

He performed General Paediatrics residency training in the Boston Combined Residency Program in Paediatrics and fellowship training in the Dana-Farber Cancer Institute/Boston Children’s Hospital Paediatric Hematology/Oncology fellowship.

He performed post-doctoral research with Dr. Tom Look at DFCI and collaborated with Dr. Kim Stegmaier at DFCI and the Broad Institute of MIT and Harvard studying the dependency landscape of high-risk paediatric solid tumours. During this time, Dr. Durbin identified cohorts of reprogramming transcription factors responsible for establishing the malignant transcriptome of high-risk neuroblastoma and rhabdomyosarcoma. In 2020, Dr. Durbin was appointed to the faculty of St. Jude Children’s Research Hospital as an Assistant Member in the Division of Molecular Oncology, Department of Oncology and the Developmental Biology and Solid Tumor Program.

Dr. Durbin’s laboratory seeks to understand how transcriptional regulatory circuitries establish malignant cell identity and derive mechanisms to perturb these for clinical benefit using conventional and novel small molecules. His work has led to the identification of several new compounds in active preclinical investigation, such as JQAD1 and iCBP4.

Dr. Durbin has received awards and grants from the National Institutes of Health/National Cancer Institute, the American Society for Clinical Investigation, the Society for Paediatric Research and numerous foundations including the Damon Runyon Cancer Research Foundation, Rally Foundation for Childhood Cancer Research, Alex's Lemonade Stand Foundation, Forbeck Foundation, CureSearch for Children's Cancer, V Foundation for Cancer Research, Hyundai Hope on Wheels Foundation and the American Society of Clinical Oncology.

Neuroblastoma

Neuroblastoma is a rare cancer that originates in the nervous system.² It starts in early immature nerve cells, called neural crest cells, that are most often found in the embryo or foetus³ and is the most common extracranial solid tumour diagnosed in children under 15 years of age, comprising around 7% of all childhood cancers⁴. It affects approximately 1,600 babies and young children every year across the EU⁵ and US⁶ (800 in each region respectively).

***Recordati** is an international pharmaceutical group listed on the Italian Stock Exchange uniquely structured to bring treatment across specialty and primary care, consumer healthcare, and rare diseases. Since 2007, our dedicated business unit for rare diseases has been "Focused on the few", with a mission to provide urgently needed therapies. We believe that health, and the opportunity to live life to the fullest, is a right, not a privilege. We want to support people in unlocking the full potential of their lives. We have fully integrated operations across research & development, chemical and finished product manufacturing through to commercialisation and licensing. Established in 1926, Recordati operates in approximately 150 countries across EMEA, Americas and APAC regions. At the end of 2023, Recordati employed over 4,450 people and consolidated revenue of € 2,082.3 million. For more information, please visit www.recordati.com*

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² Pastor ER, Mousa SA. 2019. Current management of neuroblastoma and future direction. Critical Reviews in Oncology/Hematology. 138:38-43.

³ American Cancer Society. What is neuroblastoma? Available at: <https://www.cancer.org/cancer/neuroblastoma/about/what-is-neuroblastoma.html>

⁴ Nadja C. Colon et al. Neuroblastoma. 2011; 58(1): 297–311. Available at: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3668791/>

⁵ Gatta G et al. European Journal of Cancer. 2012; 48, 1425-1433. Note: 1.8 cases of neuroblastoma per million were estimated every year in EU 27. With the current population estimated at 448 million this would equate to 806.4 patients

⁶ American Cancer Society. Key Statistics About Neuroblastoma. Available at: <https://www.cancer.org/cancer/neuroblastoma/about/key-statistics.html> [November 2020]