The International Prize for Scientific Research Arrigo Recordati 2024 Eleventh Edition

INTERNATIONAL PRIZE FOR SCIENTIFIC RESEARCH ARRIGO RECORDATI 2024

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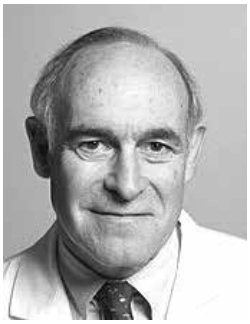

Dean for Genetics and Genomic Medicine, Professor and Chairman Emeritus of the Department of Genetics and Genomic Sciences, The Icahn School of Medicine at Mount Sinai, New York City, NY, USA

Robert J. Desnick is Dean for Genetic and Genomic Medicine and Professor and Chairman Emeritus of the Department of Genetics and Genomic Sciences at the Icahn School of Medicine at Mount Sinai.

In 1977, he joined the Mount Sinai faculty as the Arthur J. and Nellie Z. Cohen Professor of Pediatrics and Genetics, and Chief of Medical and Molecular Genetics. From 1993-2011, he was the first Chairman of the Department of Genetics and Genomic Sciences at Mount Sinai. In 2011 he became the Dean for Genetics and Genomic Medicine.

Dr. Desnick's research interests include lysosomal storage diseases (LSDs) and the inborn errors of heme biosynthesis, the porphyrias, and in particular, their treatment. His research efforts led to the Federal Drugs Administration (FDA) - and European Medicine Agency (EMA) - approval of enzyme replacement therapy (ERT) for Fabry disease (Fabrazyme) and on-going ERT clinical trials (FDA "Breakthrough" status) for Niemann-Pick B disease, both in partnership with Genzyme. In addition, he was a scientific founder of Amicus Therapeutics (NASDAQ; FOLD), which is developing oral pharmacologic chaperone therapy for Fabry disease (EMA-approved in 2016), Pompe disease, and other disorders. Currently, his laboratory is using gene editing technology to engineer gene therapy in the mouse model of Fabry disease with Sangamo Therapeutics.

For the porphyrias, he co-developed with Alnylam Pharmaceuticals an RNAi therapy for the acute hepatic porphyrias, performed the preclinical studies, and co-designed the on-going clinical trials. He also served as Principal Investigator for Clinuvel Pharmaceuticals' Phase 2 and 3 multisite clinical trials of afamelanotide, a "first-in-class" synthetic peptide for the treatment of the Erythropoietic Porphyrias, which was recently EMA-approved and pending FDA approval. He also served as the Chairman of the Scientific Advisory Committee (SAC) of Synageva Biopharma and currently serves as SAC Chair for Kiniksa Pharmaceuticals. In addition, his research includes genomics, pharmacogenomics, and personalized medicine. He has published over 740 research papers and chapters, including nine edited books. He is an elected Fellow of the American Association for the Advancement of Science and an elected member of the National Academy of Medicine.