New Dendrimeric Pyrrolidines

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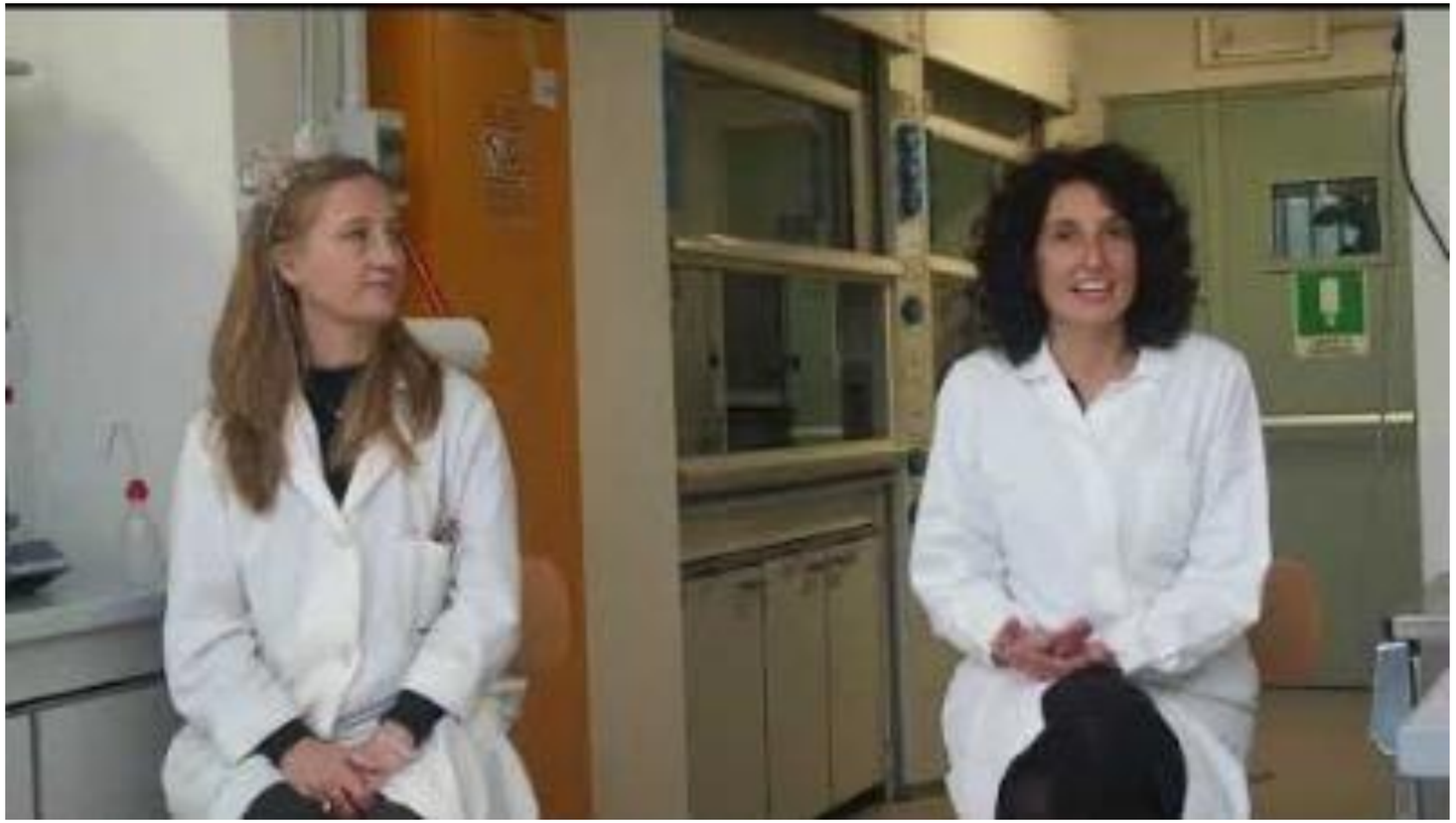
The invention

The invention proposes a new approach to rare genetic diseases for which no cures are available, only symptomatic treatments through the preparation of novel multivalent molecules active toward the human enzymes responsible for them. Lysosomal storage diseases (LSDs) are a group of rare metabolic diseases caused by deficits in lysosomal enzymes to date orphaned by treatments.

The invention is aimed at developing an effective, noninvasive therapy for the treatment of two metabolic diseases, Mucopolysaccharidosis IV (also known as Morquio A) and Mucopolysaccharidosis II (also known as Hunter's disease). Lysosomal storage diseases, a group of inherited metabolic disorders, are rare when taken individually, but are substantially numerous when taken as a whole since about one in 5000-8000 infants is affected. These diseases are "orphaned" by treatments that provide a definitive cure, but are treated only symptomatically. Our molecules are proposed as pharmacological chaperones (PCs), molecules capable of restoring deficient enzyme activity, or as stabilizers for the current marketed treatment, enzyme replacement therapy (ERT), which has various problems including poor stability of the exogenous enzyme.

Pitch



The Inventors

Francesca Cardona, associate professor at the University of Florence since 2015. PhD in Chemical Sciences in 1998. Received the "G. Ciamician" Medal from the Italian Chemical Society (organic chemistry division) for young researchers in 2006. Author of 107 papers, 1 book (RSC), 12 chapters, 1 patent, on the synthesis of nitrogen glycomimetics and new green oxidation methods.

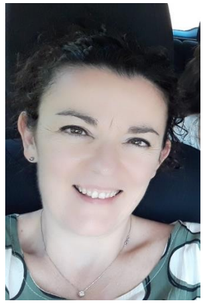
Camilla Parmeggiani, Associate Professor at the University of Florence since 2021. PhD in Chemical Sciences in 2010. LENS Associate since 2010. Received Research Award "Organic Chemistry for the Environment, Energy and Nanosciences" (Division of Organic Chemistry, SCI) in 2016. She has authored 56 papers, 3 chapters, 4 patents (h-index 29), on smart materials, nitrogen glycomimetics and new green oxidation methods.

Camilla Matassini, RTD-B of the University of Florence since 2022. Doctor Europaeus in Chemical Sciences in 2014. In 2016 she received the "Accademia dei Lincei" award in the field of Synthetic Organic Chemistry and in 2020 the SCI Research Award "Organic Chemistry for Life Sciences- Junior ". Author of 40 papers, 1 patent, 2 book chapters, on synthesis of mono- and multivalent immino sugars, gold nanoparticles, development of pharmacological chaperones.

Amelia Morrone, associate professorat the University of Florence since 2015. Head of Laboratory of Molecular Biology of Neurometabolic Diseases, AOU Meyer, Florence. PhD in Neurometabolic Sciences in 1994. Specialization in Medical Genetics in 1997 and in Biochemistry and Clinical Chemistry in 2002. Author of more than 130 papers and 2 chapters on lysosomal storage diseases and other neurometabolic diseases

Andrea Goti, Full Professor at the University of Florence since 2002. Awarded the 2020 "Adolfo Quilico" Medal by the Division of Organic Chemistry of the Italian Chemical Society. He has authored 200 publications on new green synthetic methodologies, pericyclic reactions, synthesis of heterocycles and nitrogen glycomimetics.

Giampiero D'Adamio, R&D in Fabo Tape Solutions, a leading international manufacturer of adhesive tapes. Research fellow at the University of Florence from 2011 to 2016 in the synthesis of nitrogen glycomimetics. In 2017 he obtained a PhD degree in Chemical Sciences specializing in the development of multivalent immino sugars. Author of 16 publications and co-author of one patent.



Serena Catarzi, executive biologist SOS of Clinical Pathology, San Giovanni di Dio Hospital - Florence. PhD in Clinical Biochemistry in 2003. Author of 26 publications and 1 patent.

Industrial application

The patented technology has been tested in the following case studies:

1. Pharmacological chaperones for Morquio A;
2. Combined ERT/PC therapy for Morquio A;
3. Combined ERT/PC therapy for Hunter's disease.

Pharmacological chaperone (PC)-based therapies have multiple advantages including, the possibility of oral administration, restoration of endogenous enzyme activity, and low cost of therapy; in addition, dendrimeric immino sugars can also be used in combined ERT/PC therapy as recombinant enzyme stabilizers.

Possible developments

The patent is available for outright assignment, as well as for exclusive and non-exclusive licensing. Licenses are available for the remaining term of the patent titles.

The Research Group is available for new collaborative and third-party research activities, in-depth technical investigations, scientific advice, also aimed at raising the TRL of the technology.

The TRL of the invention is 3.

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