

espeRare Foundation

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Research Areas



Tool Development

Resource



Product Development

At a Glance

- Status: **Active Consortium**
- Year Launched: **2013**
- Initiating Organization: **espeRare Foundation**
- Initiator Type: **Nonprofit foundation**
- **Rare** disease
- Location: **International**

Abstract

The espeRare Foundation is a private nonprofit organization that strives to uncover the potential of existing molecules to address severe therapeutic unmet needs in rare diseases, thus producing better chances for existing drugs to reach underserved patients.

Mission

In collaboration with patient groups and other key stakeholders, espeRare uncovers the potential of existing pharmacotherapies to address severe therapeutic unmet needs in rare diseases.

With patients groups, the espeRare model drives translational research and development activities to advance treatments for rare diseases by:

Consortium History

April 2013: Foundation created

Structure & Governance

The organization is governed by a Foundation Board whose members are chosen for their commitment and competencies in the field of rare diseases and drug development. The foundation's two other main bodies are the Executive Committee and the Head Office.

The espeRare Foundation Board supervises the foundation's affairs and the work carried out under its authority by other bodies of the foundation. The Board approves the long-term strategy, the philosophy, and general policies of the foundation.

The espeRare Executive Committee includes the member of the Head Office and representatives of the Board. Under the supervision of the Board, it is responsible for (a) development and management of the long-term strategy and (b) selection and management of the foundation's R&D programs.

The espeRare Head Office and is directly responsible for the following:

Impact/Accomplishment

Rimeporide is a proprietary compound of Merck Serono, previously developed up to clinical stage for congestive heart failure but was discontinued during Phase I for strategic reasons. Rimeporide is known as a selective sodium-proton exchanger (NHE-1) inhibitor on which detailed data on pharmacology, formulation, dosing, pharmacokinetic, and toxicology profile exists. The first focus of espeRare will be to investigate Rimeporide's potential to slow muscle degeneration in children with Duchenne Muscular Dystrophy.

espeRare is exploring the therapeutic potential of Cilengitide in focal segmental glomerulosclerosis (FSGS), a rare and deadly renal disease. Cilengitide is a small molecule drug candidate developed by the pharmaceutical company Merck Serono. This is the first in a class of α_3 integrin inhibitor that was developed until Phase III in cancer indications. The development of this drug candidate was halted in 2013 in Phase III because Cilengitide failed to meet its primary endpoint of significantly increasing overall survival when added to the current standard chemoradiotherapy regimen. espeRare is

collaborating with Moin Saleem at Bristol University to conduct research studies testing Cilengitide's therapeutic potential in experimental models of podocyte activation and motility. If demonstrated, then such a mechanism could lower proteinuria and translate into a reduced or halted decline of renal function and progression to end-stage kidney disease. Cilengitide represents an attractive therapeutic candidate for FSGS patients.

Homepage <http://esperare.org/en>

Points of Contact

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