***FOR INNOVATIVE TECHNOLOGIES TO TREAT RARE METABOLIC DISEASES***

# *This represents a Request for Proposals (RFP) from Chiesi Farmaceutici S.p.A (“Chiesi”). Please see the link to application guidelines, FAQs and a copy of the relevant RFP (i.e., Chiesi’s specific criteria for this Research Pre-Proposal) at the end of this document.*

# Project Title

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| [20 words or less] |

# Principal Investigator Contact Information

Name:

Title:

Email:

Phone:

Institution, Department, and Address:

# Abstract

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| Please include a summary of the research proposal including overall goal, hypothesis, and specific aims; clinical rationale and commercial potential (if known); and what is novel and innovative about your approach. [250 words or less] |

# a. Therapeutic Opportunity Description// [b. Platform Opportunity Description]

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| **1a.** How could the technology be applied to the treatment of rare metabolic diseases? What is the primary therapeutic area? What is the Mechanism of Action (MoA)? OR**1b.** How could the technology be applied to the treatment of rare metabolic diseases? Describe the technology and how it delivers biologics into difficult to reach organs (e.g crosses the BBB to deliver biologics into the brain). Can the technology be applied to the treatment of CNS symptoms of LSDs with CNS involvement? What is the primary therapeutic area? What is the Mechanism of Action (MoA)?**2.** How could the technology be applied to the treatment of rare metabolic disorders? Is there a particular rare metabolic disease or group of diseases to which this technology would be specifically appropriate? [*please consider that the therapeutic area of interest for this call is rare metabolic disorders, for example Inborn Errors of Metabolism, including Lysosomal Storage Disorders.*]**3.** What is the stage of development of the program? [For example: *Discovery, in vitro/in vivo preclinical validation, etc*]**4.** What is the therapeutic modality? [For example: *peptides, proteins, mAbs, nucleic acids, etc*] Please describe the manufacturing process and your thoughts on scalability.**5.** Provide a brief description of the target/pathway and link to human disease. |

# Scientific Background and Rationale

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| **1.** Summarize the hypothesis, background science and rationale, and work completed to date.**2.** Provide key evidence/data to support the hypothesis, this may include *in vitro* and *in vivo* models and preclinical proof of concept.**3.** Describe the project within the current state of the art (including scientific literature) to highlight what is novel and innovative. Please also include uncertain or unknown aspects you intend to investigate in your project proposal. |

# Research Plan and Milestones

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| **1.** Provide a description of the research plan to be carried out to reach your goals (objectives and specific aims). Please include technical methods, experimental design, and estimated timeline. |

# Budget, Resources and Funding

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| Awardees can request up to 2.8M SEK total funding, including all direct or indirect costs. Term of funding should not exceed two years.**1.** Specify what the award money would be spent on (including resources, if any).  |

# IP and Legal Considerations

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| **1.** Have you previously disclosed this technology to your technology transfer office? If yes, who is your Case Manager (please include email address). Have you disclosed this technology in any form (for example: poster, congress, article, thesis)?**2.** To the best of your knowledge, what is the prior art in this space (publications, patent applications, and issued patents)?  |

# Research Team

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| 1. Provide a brief biographical sketch of the principal investigator and a list of his or her key publications. An NIH format biosketch is acceptable.
2. List other team members (if any), including collaborators, and describe their roles in the project. Please provide a brief biographical sketch of the co-investigators.
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# Co-Investigators

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
|  | First Name | **Last Name** | **Title/ Department/ Institution** | **Role** |
| Co-I 1 |       |       |       |       |
| Co-I 2 |       |       |       |       |
| Co-I 3 |       |       |       |       |

Once completed, please submit your Pre-Proposal by email to AcademicRFP@Chiesi.com by **October 31, 2024**.

Full program details, including guidelines, criteria and FAQs can be found here: <https://www.chiesipharma.se/FoU>