IHI Call Days | Call TS2 (SO2)

Advancing processing, scale-up and clinical readiness of RNA delivery platforms

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Challenges and objectives

- **What problem(s) are you trying to solve**

There are many new RNA candidates being discovered and being used in novel applications. However, there are still barriers to getting these candidates into clinical trials, due to factors such as cost of goods, requirement for cold chain storage or stability during alternative administration routes, lack of understanding around fundamental structure, challenges on scale-up and regulatory guidance. Additionally, these new candidates encompass a variety of RNA types, designs and delivery platforms, which can require bespoke development from the bench to the clinic. Whilst the challenges are openly discussed, development in these areas is currently fragmented, in part due to a heavily contested IP space.

- **Which IHI specific objective(s) are you addressing?**

This project would address the high economic impact of the disease for patients and society, by reducing costs associated with RNA delivery processes, and have a transformational nature of the potential results on innovation processes, by reducing time and barriers to RNA drug development – enabling products to get to patients faster across a wide range of diseases. The outputs from this project can then be transferred into RNA industrial processes for multiple therapeutics.

- **Which unmet public health need are you addressing?**

The use of RNA as both a therapeutic and a vaccine has rapidly expanded since the potential was unlocked on the global scale during the Covid-19 pandemic. RNA technology is now being used to prevent and treat a much larger portfolio of diseases, such as infections, cancer and rare genetic diseases. It is widely seen as the technology to treat previously untreatable diseases.

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Your approach to solve the problem

- The initial focus will be around RNA-lipid nanoparticles, specifically those with freedom to operate. However, this project will require a study of a variety of different RNA-lipid nanoparticles, RNA types, designs and other delivery platforms to ensure that the solutions can be applied across the field.
- This project will look to develop characterisation techniques, to provide fundamental understanding of these systems, and to define specifications that directly correlate to in vivo performance, to accelerate the drug development process.
- It will also look at how to speed up screening of candidates at small scale, and how they correlate to scaled-up processes. The scaled-up processes will be developed to ensure they are scalable across a broad range of batches, intensified, high yielding, clinic-ready and able to support different RNA therapeutics which require alternative stability or administration technologies.
- Across the project, there will be a focus on the solutions being more sustainable (e.g., removing cold chain storage, becoming solvent-free) and cost-effective.
- Regulatory guidance will be given throughout the project, to develop clearer guidance and understanding for the industry on RNA therapeutics.

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Is your project suitable for IHI?

- As the RNA Therapeutics space is currently fragmented, this proposal aims to bring the industry together to work collaboratively on a solution to benefit everyone, whilst not developing a specific drug candidate for commercialization to avoid complex IP situations. Currently, the knowledge/expertise required to make this proposal successful sits across the industry, including with many academics.

- This project would bring together early-stage research (academics), a broad range of test cases (SMEs), commercial guidance (industry) and regulatory advice (e.g., MHRA), to facilitate success.

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Outcomes and Impact

- This project aims to increase the speed of RNA drug development, across multiple RNA and delivery platforms, through:
  - Improved understanding of what early-stage characterisation can be indicative of in vivo performance, and can be used as consistent metrics throughout development and scale-up
  - Cheaper, higher yielding and more sustainable development platforms, from small-scale screening to large scale manufacturing
  - Improved processes to support the removal of cold chain storage and alternative administration routes
  - Clear regulatory guidance to steer product development
- By having academics, SMEs, large scale industry and regulatory authorities involved, the solutions can be directly implemented across the industry, to have an immediate impact across the drug delivery pipeline.
- This project would allow Europe to be pioneering the way for RNA therapeutics, by breaking down the current barriers and facilitating quicker delivery timelines for these therapies to maximize the benefit to patients.
- The project will facilitate quicker development of novel RNA drug candidates to bring treatment of previously untreatable diseases to patients faster.

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Expertise and resources

- We have:

CPI has extensive experience in process development and scale-up support for RNA and RNA delivery platforms, including lipid nanoparticles. This is furthered by our Centres of Excellence in both RNA and intracellular drug delivery. In addition to our technical expertise, we have multiple teams that enhance our offer, including our MSAT team who have expertise in CMC development, our process engineers who can provide manufacturability support and perform technoeconomic and life cycle analysis, and our automation and digital teams who can increase speed and quality of development.

- We are looking for:
  - Academics to provide development support and expertise
  - SMEs to provide RNA and delivery platform test cases
  - Large industry partners to support the need and provide guidance
  - Regulatory authorities, e.g., MHRA, EMA, to provide regulatory support

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