

## opn2EXPERTS - Intracellular delivery of therapeutic proteins

### How would you propose to deliver therapeutic proteins such as antibodies intracellularly?

If you have an answer to our question, please submit your proposal for collaboration via the [opn2EXPERTS web page](#).

#### What is the context of the problem that we would like to solve?

Many disease-relevant proteins are enzymes, transcription factors or part of signaling pathways and are located in the intracellular compartment. Small molecules have a great track record in interfering with intracellular target proteins and thereby execute a therapeutic effect. However, small molecules are mostly dependent on defined binding pockets in the target protein, limiting the druggable target space. In contrast, biologics, such as antibodies, can also bind with high affinity and specificity to planar target domains and thus can address different targets than small molecules. Unfortunately, biologics display poor cellular membrane permeability and cannot target intracellular proteins easily. Hence, technologies are required to deliver therapeutic proteins, such as antibodies intracellularly. Utilizing such delivery technologies opens up the intracellular target space for biologics and enables new therapies of diseases, in particular therapy of cancer.

#### What potential solutions could be in scope?

The following potential approaches to answer our question include, but are not limited to, the following:

- Delivery technologies that are ideally based on proteins, peptides, or antibodies.

- Delivery technologies that fit to industry standard biologics manufacturing platforms.
- Technologies that deliver therapeutics to tissues at a concentration that is therapeutically relevant and potentially scalable to human use.
- Delivery technologies that are able to direct cargo to specific subcellular compartments, including the nucleus.
- Delivery technologies that rely on nanoparticles or dendrimers are not the primary focus of this call but would be considered.

Of note for this question:

- Delivery technologies, which enable tumor cell/tissue specific redirection of the biologic in addition to intracellular delivery, are preferred.
- Proposals including data on *in vivo* proof of concept are preferred.

### **What potential solutions would be out of scope?**

- Proposals that rely on specific formulations using exosomes or liposomes.
- Proposals that rely on viral delivery based technologies

### **What benefits do we offer to you in exchange for having submitted a solution?**

We are open to all proposals that can fully or partially meet our requirements.

If your project is selected, you will have the opportunity to directly collaborate with the Biotherapeutics Discovery Research (BTD) team of Boehringer Ingelheim. You can expect appropriate funding for the prospective collaboration period. Your exact funding request should be outlined in your proposal. As a framework, we suggest that your initial funding request is clearly structured in milestones including proposed Go/ No-Go criteria. Ideally, the initial milestones resulting in Go/No-Go decisions will not exceed 200,000 euros per submitted project in total. Please note that additional budget will be made available if the milestones and Go decision points are met. We set ourselves the goal to complete all evaluations within four weeks after the deadline of the submission period (i.e. evaluation feedback by end of November 2020).

The opportunity for a funded stay at Boehringer Ingelheim for technology exchange / training is potentially available, as is the availability of custom biological tools and reagents. It is also possible to run specific experiments at BI.

Our collaboration agreement will provide full transparency about each partner's rights & obligations (including intellectual property rights). As part of the agreement, you will be encouraged to publish following the collaboration agreement (to be negotiated in good faith). There is also the possibility to choose the name of the technology.

In order to foster the highest degree of open innovation possible, we plan to announce the winner(s) publically and feature them on opnMe.com and our social media channels. We would guide you through this process and, as part of it, we would kindly ask for your upfront consent, in case our scientific jury had selected your answer. An alignment on a common communication strategy is supported.

## **What are the key success criteria on which we base our selection for the best answer?**

We are seeking research collaboration proposals that contain:

- A well-structured proposal outlining a new and compelling scientific idea,
- A novel, testable working hypothesis distinct from those previously published,
- A well-structured experimental plan that will be used to test the hypothesis, and potentially existing data,
- Inclusion of key decision points [Go/No-Go criteria] and a milestone plan including an outline about budget requirements for the work until the first Go/No-Go milestone step,
- Proven track record in the required field of expertise,
- Outlining the technical feasibility of the innovative proposed approach,
- Ability to implement the outlined solution as part of a scientific collaboration project including access to a laboratory.

## What information should be included in your answer submission?

Please use our answer submission template to provide a 2-3 page non-confidential proposal (available for download on the following [site](#)).

If confidential data exists that would strengthen the proposal, please indicate that confidential information is available to share under a Confidential Disclosure Agreement (CDA). If we find the non-confidential concept proposal sufficiently interesting, we will execute a CDA for confidential discussions.

## Anticipated Project Phases or Project Plan

- Phase 1 Please complete your submission.
- Phase 2 We commit to review your proposal within four to six weeks after submission and notify you about the outcome of our review after this timeline.
- Phase 3 We may propose a web-conference from our end to discuss your proposal and potential next steps in case of a positive review outcome. At this meeting you will also be encouraged to bring up your own questions and/or suggestions. As an outcome of this meeting, we may decide to proceed with a mutually agreed collaboration proposal.

## Submitting a collaboration proposal

- Check the outline of the [opn2EXPERTS Delivery Technology question](#) on opnMe or alternatively,
- Click the “I have an answer” banner to access the collaboration submission template.
- Follow the instructions upload your submission document (requires login or registration).
- The upload allows you to attach additional application files if you want to.
- You will be able to access your final submitted collaboration proposal in your personal dashboard and follow its review status.
- Please also visit the [FAQ section](#) on opnMe.com to learn more about our opn2EXPERTS program.