



Launch of COMPACT

An Innovative Medicines Initiative Project for Biopharmaceuticals

Academia, Biotech and the Pharmaceutical industry have joined forces to deliver the next generation of biologics-based medicines

Brussels, Frankfurt, Paris, Utrecht, Ware – November 29, 2012 - COMPACT (Collaboration on the Optimisation of Macromolecular Pharmaceutical Access to Cellular Targets), a public-private consortium funded by the Innovative Medicines Initiative (IMI), announced today the launch of a 30 million Euro project focussing on improving the delivery of biopharmaceuticals. Academic institutes, biotechnology companies and the pharmaceutical industry have joined forces to tackle the major delivery and targeting bottlenecks in the development of novel innovative medicines based on biological macromolecules, such as proteins/peptides and oligonucleotides.

The COMPACT consortium kick-off meeting was held under sponsorship of Sanofi Germany in November 2012 in Frankfurt and was attended by a large team of consortium participants representing the leading European experts from 14 academic institutions, two biotech companies and seven pharmaceutical companies. The consortium's board of directors includes Ekkehard Leberer from Sanofi as scientific coordinator, Steven Hood from GlaxoSmithKline as his deputy, Enrico Mastrobattista from the University of Utrecht as academic coordinator and Nathalie Piton from Sanofi as consortium manager. "The COMPACT consortium offers excellent possibilities to join forces to address a major challenge in the development of innovative biotherapeutics by combining academic fundamental research and applied drug development in the pharmaceutical industry," said Ekkehard Leberer. Enrico Mastrobattista commented: "I am very excited about this unique public-private partnership between major players in the pharmaceutical field to work on the problem of delivery of biopharmaceuticals. Only with collaboration at this scale will we be able to tackle some of the urgent problems that hamper the development of candidate biopharmaceuticals into useful medicines."

Most biopharmaceuticals currently on the market are recombinant proteins which are parenterally administered. These would benefit from patient-friendly routes of administration and more effective means to target their delivery across major biological barriers, such as the blood brain barrier (BBB), and open important new avenues for the treatment of major hitherto untreated diseases. Moreover, new classes of biopharmaceuticals (e.g. oligonucleotides such as siRNAs, and therapeutic peptides) with specificity for intracellular targets hold great promise but await the advent of efficient tissue and cell delivery systems before their potential can be translated into therapeutic products. Solving the major medical challenge of targeted delivery of biopharmaceuticals will pave the way for better, safer and more innovative medicines in areas of major unmet medical need like cancer, diabetes, Alzheimer's disease and rare genetic diseases.

About COMPACT:

COMPACT represents Europe's frontrunners in pharmaceutical sciences, nanotechnology, biology, chemistry, engineering and bio-imaging who will work on the challenges of biopharmaceuticals delivery. The main objectives are to identify and understand transport pathways across major biological barriers and cell membranes that can be harnessed for delivery of biopharmaceuticals, and to construct, characterise and pre-clinically validate novel formulations of biopharmaceuticals for their non-invasive delivery with an emphasis on oral, airway and (trans)dermal routes of administration and for transport across the blood brain barrier after systemic administration. This will be achieved by setting up sophisticated *in vitro* models of biological barriers and appropriate animal models to identify and exploit novel cell pathways for effective delivery of biopharmaceuticals. In parallel, COMPACT will alter the biopharmaceuticals either by chemical or biological engineering or by nano-formulations to enhance their cellular uptake or transport over biological barriers. To be able to follow the transport and stability in the various stages of the delivery process, advanced imaging techniques will be utilised.

The unique feature of this consortium is the alliance of experts in different research fields that complement each other in this challenging task. It is only the concerted action of such an interdisciplinary consortium that can make a difference in finding constructive solutions to the problem of poor bioavailability and targeted accessibility of biopharmaceuticals.

The COMPACT team, coordinated by Sanofi, GSK and Universiteit Utrecht is working on finding ways to bring biopharmaceuticals to their target while preserving their effectiveness. The five year scientific program will deliver:

- Detailed elucidation of cellular uptake and processing mechanisms;
- Understanding of variables which determine distribution of biological macromolecules in tissues;
- Organ and tissue specific nanotechnology-based delivery methods for biological macromolecules; and
- Delivery strategies for biological macromolecules via local and non-injectable application routes.

COMPACT participants are Sanofi-Aventis Deutschland GmbH, Universiteit Utrecht, University of Copenhagen - Faculty of Health and Medical Sciences, Helmholtz-Institute for Pharmaceutical Research Saarland (HIPS) - Helmholtz Centre for Infection Research (HZI), Cardiff University, Stockholms Universitet, Norges Teknisk-Naturvitenskapelige Universitet NTNU, Universität Wien, Ludwig-Maximilians-Universität München, Universitaet Zürich, Universiteit Gent, Pharmacoidea Development and Service Ltd, Bioneer A/S, Helsingin Yliopisto, Universiteit Leiden, The Chancellor, Masters and Scholars of the University of Oxford, Glaxosmithkline research & development ltd, AbbVie Deutschland GmbH & Co. KG, Novo Nordisk A/S, Merck KGaA, Boehringer Ingelheim International GmbH, Pfizer Limited.

For further details – please visit: www.compact-research.org

About IMI:

The Innovative Medicines Initiative (IMI) is the world's largest public-private partnership in healthcare. The European Union contributes €1 billion to the IMI research programme, which is matched by in-kind contributions worth at least another €1 billion from the member companies of the European Federation of Pharmaceutical Industries and Associations (EFPIA). IMI is improving the environment for pharmaceutical innovation in Europe by engaging and supporting networks of industrial and academic experts in collaborative research projects.

IMI currently funds 30 projects, many of which are already producing impressive results. The projects do not aim to develop new medicines as such, but focus on new methods and tools that will enable the entire sector to accelerate the development of safer and more effective treatments for patients. A comprehensive overview of ongoing projects is available at www.imi.europa.eu.

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