Academic drug development across Europe, regulatory challenges and learnings

RSNN session @ FIGON-DMD/EUFEPS 2022 – Key Takeaways

On the 2nd of June 2022 the RSNN hosted a parallel session during the FIGON & EUFEPS European medicines days (www.figondmd.nl) in the Stadsgehoorzaal in Leiden. The session was chaired by Sini Eskola (EFPIA) and speakers from different backgrounds were present.

In three presentations, followed by a plenary discussion, the view from academia, industry and regulator were discussed with regard to academic drug development. Furthermore, speakers debated about the way academic drug development can potentially have added value and the hurdles to overcome for academic drug development in the route to market authorisation and beyond.

Chair:
Sini Eskola - *European Federation of Pharmaceutical Industries and Associations (EFPIA)*

Speakers:
Teun van Gelder - *Clinical Pharmacology, Leiden University Medical Centre (LUMC)*
Henk Jan Out - *Pharmaceutical physician/consultant*
Ralf Herold - *Task force Regulatory Science and Innovation, European Medicines Agency (EMA)*

ACADEMIC DRUG DEVELOPMENT ACROSS EUROPE, REGULATORY CHALLENGES AND LEARNINGS

Basic science in an academic setting, in terms of looking for new targets and new compounds, is traditionally the kick-off for a drug life cycle. After translational research with a promising lead, industry has a chance to take over from academia. Industry would then conduct clinical trials or use clinical trials sponsored by academia, which ideally will lead to registration of the drug.

The Leiden University Medical Centre (LUMC) invests in the field of academic pharma, under the umbrella of the research theme *'Academic Pharma 2.0: Innovative drugs from bench to bedside'*. Academic Pharma has the goal to support academic drug development of innovative medicinal products and repurposing already existing medicinal products. Also, the University Leiden has allocated funds for translational drug discovery and development, and has joined forces with LUMC.

In the view of some of the speakers, supporting academic drug development is aimed by creating awareness of: (im)possibility of protecting intellectual property, impact of regulatory issues, and the possibility of gathering scientific advice in an early stage. The average academic researcher mainly has the focus on science, innovation, and top publications, but less on a drug development plan. Current practice shows that even if academic researchers are aware of the possibility to gather regulatory advice in an early stage, there is still quite some reluctance because of lack of experience in this field.

Academic drug development can be of great added value, for example when there is an unmet clinical need, such as in the rare diseases area. It has a non-profit basis and includes new chemical entities as well as repurposing aiming to protect this compound for an external party which could lead to an unnecessary increase in the price. Together with other universities and the FAST initiative (Future Affordable & Sustainable Therapies), LUMC's Academic Pharma hopes to generate a national expertise center to overcome the hurdles to be taken by academia in the road to market authorization and afterwards. So far, Academic Pharma has reached several successes, including but not limited to the production of 3,4-diaminopyridine for Lambert-Eaton syndrome and a gene therapy for SCID (the first-time stem cell gene therapy of Dutch origin has been administered to a patient).

In parallel, some hurdles experienced in academic drug development were found to be difficult in regulatory medicinal product lifecycle maintenance and pharmacovigilance after

registration. Compared to industry, academia is variably organized in global clinical networks, which are needed to conduct multinational clinical trials, and often lack substantial funding to do regulatory applications, both new and maintenance variations. Therefore, one might question the rationale and opportunities for academic drug development.

Industrial clinical development is a process that takes into account a broad spectrum of considerations including medical need, potential for differentiation compared to other products, probability of scientific success and reimbursement potential, speakers reminded. Currently new medicines mainly originate from industry and when taking expenditure into consideration it is hard to imagine that this model would be desirable to be emulated with public money (Wouters et al. JAMA 2020; 323:844-853). Drug development is risky and requires heavy investments upfront without guarantees for success in getting the medicine to the patient.

From the regulators' perspective, the European Medicines Agency (EMA) is systematically engaging with stakeholders and has built a successful framework with academia. One of the goals is to support academic developers and support for translating academic research into novel methodologies and medicines. The STARS initiative (Strengthening Training of Academia in Regulatory Science) is a collaboration between 21 regulatory partners from 18 countries, including the EMA, that generated recommendations how to improve the regulatory knowledge, awareness, and skills of academia interested in contributing to medicine development. In addition, active engagement of the developer is necessary to evolve the regulatory framework itself. This is sought for example by innovative training models and by providing scientific advice for and with academia.

In conclusion, key points that emerged from the session are:

- Academic drug development can be of added value for example when it comes to high unmet medical needs for a small population that industry is not capable, or does not have the needed incentives, to fulfill.
- The aim of academic drug development is explicitly not to be competitive to industry but to be complementary.
- One of the big hurdles for academia to be taken is how to increase the knowledge on regulatory science.
- The EMA is extending its framework for and with academia to improve engagement and involvement with regulatory activities and medicine development.
- Industry can play a role in sharing knowledge with academia about drug development, regulatory science, and regulatory procedures.