Q&A SESSION WITH CHRISTIANE NIEDERLAENDER

CHRISTIANE NIEDERLAENDER, Senior Quality Assessor and Unit Manager, Biologicals, **MHRA**

Christiane Niederlaender is a Senior Quality Assessor and Unit Manager, Biologicals with the Medicines and Healthcare Products Regulatory Agency (MHRA) in the UK, where she has worked since 2011. She is also the Acting Unit Manager of the Biologicals Unit there. Christiane has experience in the assessment and regulation of all classes of biological medicinal products and has been responsible for the regulatory review of many new biological products including recombinant proteins, blood products and several Advanced Therapy Medicinal Products. She is the current UK representative at the Committee for Advanced Therapies (CAT) at the EMA. Prior to joining the MHRA, Christiane has worked for several years at the UK Human Tissue Authority (HTA) in the regulation of Tissue and Cell Therapies under the European Tissues and Cells Directive. Christiane received a Ph.D. in molecular developmental neurobiology from Kings College London and has spent several years researching and publishing in the area of cancer, development and neurobiology before taking a law degree and joining the regulatory profession.

What have you been working on at the MHRA?

As a Senior Quality Assessor in the Biologicals Unit of the MHRA, I have been dealing with the licensing and assessment of all types of biological medicinal products. At the same time, I frequently provide scientific advice during the development phase of biological medicines. I have specialist expertise in advanced therapy medicinal products (ATMPs) and have been a member of the Committee for Advanced Therapies (CAT) at the EMA. I have also been regularly involved with the EMA's Biologics Working Party (BWP).

ATMPs consist of cell based and gene therapy products which represent cutting edge science, from this stems my general interest in novel biotherapeutic products. I have been actively involved in the MHRA's Innovation Office (IO) service which provides regulatory advice and guidance to organisations developing innovative medicines, medical devices or novel manufacturing processes. I have also been involved in providing advice via the Innovation Task Force (ITF) at the EMA which provides a similar service for very early stage products and technologies.

In your experience, what have been the key challenges in the regulation of novel biotechnologies?

Although biotechnological medicines, such as enzymes and antibodies manufactured using recombinant DNA technology, are now regarded as established, it is important to bear in mind that they represent comparatively new technologies in the field of medicines. Hence regulators of biological medicines have already gained experience in adapting the regulatory environment to the challenges that new developments in biotechnology pose.

Developers of new biotechnologies are not normally familiar with the regulatory landscape and one of the first hurdles to overcome is to identify where in the framework a product fits. It could be a medicine, a device, a diagnostic or none of these and be simply a manufacturing aid. The precise application of a product may not be known during early development and at that point many technologies may fit potentially under a variety of different regulatory frameworks, so developers need to bear in mind the different scenarios that could eventually apply.

For the regulators themselves it is important to be kept abreast of new developments by companies which helps identify potential problem areas early on.

What potential do you think synthetic biology has for healthcare as a whole?

Synthetic Biology is such a wide field that it is impossible to underestimate the promise for healthcare and medicines, the limits are only the creativity of researchers in the field.

There are exciting developments in almost every aspect of them. Therapies that are closer to the clinic at this point are the resurgence of engineered therapeutic microbes, where there is potential to address a very wide range of diseases from cancer to infections and inherited disorders. In an era of perpetual donor shortage for organ transplants, bioprinting and the growth of organs in the laboratory also has to be ranked as on of the key hopes associated with the field. Of course there is great promise from synthetic biology for the development of regenerative medicines in general.

Apart from directly applicable approaches, the expansion of biology-based tools that can be employed in research will speed up the discovery of new treatments immensely.

What do you intend to get out of attending the Synthetic Biology Congress in London in November?

I look forward to this meeting as a great opportunity to learn about the latest developments in biotechnology and related areas, and based on the programme so far there should be plenty of opportunities to learn something new. I also want to get a more in depth understanding of what researchers see as the key issues for new products in this field, when it comes to bringing their technologies into clinical use.

I am particularly excited to learn about the latest trends in using CRISPR gene editing, which has shown such promise in recent years. I also have a special interest in leaning about applications for computational approaches and AI in biotech. As regulators, we have identified that in respect of these there is a need to consider how we integrate them into the regulatory framework as they can be used in a way that has significant impact on the quality of manufacture of biological medicines.