Newsletter

May 2018 – XIII PPH plus Expert Workshop – Part II of II

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Biosimilar Safety Surveillance – Points to Consider in Orphan Drug Development – Risk-based Quality Management

This bulletin completes the summary of the XIII Interdisciplinary Drug Development Expert Workshop on "Successful Drug Development under Cost Constraints and Complex Regulations" (Frankfurt Biotech Innovation Center - FIZ, Frankfurt am Main, 20 April 2018) with the focus on three current topics: Adverse Drug Reactions and accurate identification of specific biosimilar product administered, clinical development and regulatory specifics of orphan indications, and risk-based quality management after effective date of the ICH E6(R2) guideline.

Product-Specific Safety Surveillance of Biologicals/Biosimilars

Dr. med. Jürgen-Hans Schmidt, Director Risk Management, Sanofi-Aventis Deutschland GmbH, expanded on the criticality of the detection of potential differences in safety signals between a biosimilar, its reference product, and other biosimilars for the following underlying reasons:

- Possible new/unknown risks or other incidences or severities of known risks may occur due to a different manufacturing technology.
- Switch between biosimilar and original may lead to increased immunogenicity risks and makes the adjustment of the dosages for similar efficacy necessary.
- Fluctuations in the efficacy may lead to side effects (e.g., insulin: hyper-/hypoglycemia); real long-term experience with biosimilars does not yet exist.
- Retraceability of the drug applied is very restricted (details rarely available, e.g. batch numbers).

Full presentation (primarily in German language) available here.

Points to Consider in Orphan Drug Development

PPH plus gladly welcomed back Dr. med. Guido Würth, Head of Clinical Development and Medical Affairs, MorphoSys AG, Planegg, Germany, as expert speaker of this annual event for the third time.

The key messages of Dr. Würth's presentation were:

- One out of 17 EU citizens suffers from a rare disease, i.e., presumably 30 out of 510 million people are affected. This translates into a huge unmet medical need.
- Six thousand rare diseases are known but only 1,900 medicines enjoy an orphan drug designation.
- Approx. 140 compounds for the treatment of 116 rare diseases have passed all hurdles to successful EU market authorization, primary reasons being difficulties in reaching a sufficient number of patients recruited globally as needed for pivotal studies, as well as the uncertainty of an adequate return on investment.
- Development incentives have been in place in the US since 1983 (Orphan Drug Act) and in Europe since 1999 (Orphan Drug Regulation).
- About 13 % of the worldwide total prescription costs are currently assigned to the treatment of patients with approved orphan drugs. The 10 top selling orphan drugs in the EU are expected to grow from 2016 to 2022 at a CAGR of 8.9 %.

Full presentation (in German language) available here.

Risk-based Quality Management (RBQM)

Dr. Artem Andrianov, CEO of Cyntegrity Germany GmbH, a solution provider for de-risking clinical trials, was the final speaker prior to a lively and inspiring discussion triggered by challenging questions and comments stemming from a wealth of professional experience of the attending interdisciplinary audience.

Dr. Andrianov alluded to the software-supported methodology of risk assessment, control and detection, the required setting of quality tolerance limits for clinical trial parameters leading to a decrease of systematic errors, i.e., errors that matter, over time. He referred to the benefit of early warning and mitigation mechanisms that come in sync with performance, quality and risk control.

Quality through enhanced compliance and optimized trial outcomes come at a cost. No 'quick fixes' but solid quality improvements over time are the return on investment.

Full presentation available here.