Industry Perspective

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21 & 22 August 2017
Interventional trial status in the US

*Industry sponsored*

Search terms include

- Study type: “interventional”
- Study phase: “Early Phase 1; Phase 1; Phase 2; Phase 3; Phase 4”
- Funder type: “industry”

Source: [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (Site accessed 09 Aug 2017)
**Biotechnology Innovation Organization Preclinical Safety Committee (BioSafe)**

- BIO members are involved in the research and development of innovative healthcare, agricultural, industrial and environmental biotechnology products
- BioSafe serves as a resource for BIO staff and member companies by identifying and responding to key scientific and regulatory issues related to the preclinical safety evaluation of biopharmaceutical products
  - Over 30 companies
  - Develop white papers, responses to regulatory guidance, and expert topic meetings
International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use (ICH)

• Brings together regulatory authorities and the pharmaceutical industry to discuss and achieve consensus on scientific and technical aspects of drug registration

• Mission: To achieve greater harmonization worldwide to ensure that safe, effective, and high quality medicines are developed and registered in the most resource-efficient manner

- S5(R2): Detection of Toxicity to Reproduction for Medicinal Products & Toxicity to Male Fertility
- S5(R3): Revision of S5 Guideline on Detection of Toxicity to Reproduction for Human Pharmaceuticals
- S6(R1): Preclinical Safety Evaluation of Biotechnology-Derived Pharmaceuticals
- S9: Nonclinical Evaluation for Anticancer Pharmaceuticals
- M3(R2) & M3(R2) Q&As: Guidance on Nonclinical Safety studies for the Conduct of Human Clinical Trials and Marketing Authorization for Pharmaceuticals
ILSI Health and Environmental Sciences Institute (HESI) Developmental and Reproductive Toxicology (DaRT)

- Provides a forum where scientists from industry, government and academia can exchange information and initiate activities to:
  - Advance science related to DART
  - Develop consensus on the appropriate use of experimental data for human health risk assessment
  - Areas of scientific focus include:
    - Developmental toxicology
    - Male fertility
    - Female fertility
    - Juvenile toxicology
    - Multi-disciplinary guidance as it relates to DART issues
Impactful publications (http://hesiglobal.org/developmental-and-reproductive-toxicology-dart/) include:

- Scialli AR et al. Potential Seminal Transport of Pharmaceuticals to the Conceptus. Reproductive Toxicology. 2015.
Planned and held US- and EU-focused workshops that provided practical training on the requirements related to the FDAs Pregnancy and Lactation Labeling Rule

Satellite meeting of the European Teratology Society

  - To achieve a consistent and accurate classification in regard to developmental toxicity, in particular within the REACH, biocides or pesticide regulations
  - Topics
    - The interpretation of developmental toxicity studies (developmental effects in the presence of maternal toxicity)
    - The weight of evidence in determining a classification
Public-Private partnerships

PROTECT (Pharmacoepidemiological Research on Outcomes of Therapeutics by a European Consortium)
https://www.imi.europa.eu/content/protect

Consortium of 34 public and private participants (2009)

- To address limitations of current methods in the field of pharmacoepidemiology and pharmacovigilance
- To enhance the monitoring of the safety of medicinal products
- To better evaluate and communicate benefit-risk profiles throughout lifecycle
- Assessed modern means of collecting data on medication, lifestyle and risk factors directly from consumers in 4 countries (2066 pregnant women)


“Direct to consumer studies offer important benefits in obtaining data not found in prescription drug registries and electronic health records, and these type of studies will be most informative when combined with selected data from other sources to validate clinical outcomes of interest, and to corroborate most important exposures.”
Public-Private partnerships (1)

ConcePTION (Continuum of Evidence from Pregnancy Exposures, Reproductive Toxicology & Breastfeeding to Improve Outcomes Now)

IMI Consultative Workshop

- 15 Dec 2016 (Brussels) Safe use of medicines during pregnancy/lactation
- Opportunity for collaboration stemming from:
  - Expectations of the public and regulators for better information on risks associated with disease and medication are rising (new pregnancy and lactation labeling guidelines in EU are anticipated)
  - New data analytic approaches and data sources allow efficient access to and learning from much larger pregnant populations
  - Growing consensus across stakeholders that collaboration is the way forward to address the issue
The ConcePTION project proposal addresses:

- The unmet need for a science and data driven approach to define the standards for generating data on medicines used during pregnancy and breastfeeding.
- To provide better information to both the HCP and the women who are or want to become pregnant or breastfeed.

- 15 EFPIA member companies
- Call launch anticipated in October 2017
European Forum for Good Clinical Practice (EFGCP)

- Non-profit organization established by and for individuals with a professional involvement in the conduct of biomedical research
  - Purpose: To promote good clinical practice and encourage the practice of common, high-quality standards in all stages of biomedical research
  - Position statement (Mar 2014)

“As part of the responsible transition for a woman who falls pregnant during a study, the EFGCP strongly recommends:

That she meets the researchers responsible for her care in the study, along with an appropriate counsellor to discuss:

- Implications for her and her baby,
- Whether or not she should remain in the study,
- What further data collection will be done,
- and future therapeutic management and prenatal care.

That her study medication is unblinded and shared with her, unless she takes an informed position not to unblind.”
DIA Medicines in Pregnancy Forum (2014)

➢ To gain multi-stakeholder alignment on key ethical questions related to research in pregnant women, and to provide insight on how best to facilitate discussion and action

• *When is it ethically appropriate to include or unethical not to include pregnant patients in clinical studies, and how can ethical barriers be addressed?*

• *What are the most appropriate methods to collect and share data on medication use in pregnancy, and what is the best process for sharing such information?*
Multi-stakeholder forums

Resulting publications


Thank You!

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