



## CRED: Understanding Clinical Development Programme

14-15 October 2025

Day one

Chairperson: Steve Pinder, Envestia Ltd

Time	Activity	Speaker
09:20	Registration and Coffee	
09:30	Introduction to TOPRA	
09:35	<b>Welcome and Introduction</b> <b>Clinical Development in Context</b> <ul style="list-style-type: none"> <li>• Target product profile</li> <li>• Use 10-year development diagram, say where everything fits in</li> <li>• Why are clinical data needed?</li> <li>• Relevance of preclinical data</li> <li>• Definitions of Phases I, II, III and IV.</li> <li>• Clinical development strategy and the Clinical Development Plan</li> <li>• Sources of advice and timing</li> <li>• Need for a PIP</li> </ul>	<b>Steve Pinder</b> Envestia Ltd
09:50	<b>Clinical Pharmacokinetics</b> <ul style="list-style-type: none"> <li>• To see how the drug is handled in man</li> <li>• To understand the basic parameters used to describe the PK of a drug</li> <li>• To understand the importance of PK in drug development               <ul style="list-style-type: none"> <li>○ Describe the different processes involved in Pharmacokinetics: absorption, distribution, metabolism and excretion</li> <li>○ Define the PK parameters which describe each process, e.g. Cmax, t<sub>1/2</sub>, AUC, Volume of distribution, Clearance, Bioavailability etc, and their relevance</li> <li>○ Discuss multiple dosing and non-linear kinetics</li> <li>○ Understand the importance of metabolism including,                   <ul style="list-style-type: none"> <li>▪ Drug metabolising enzymes,</li> <li>▪ Importance of ensuring main metabolites in man are similar to those produced by preclinical toxicology species</li> </ul> </li> <li>○ Discuss generation of PK data throughout the different phases of Drug development including                   <ul style="list-style-type: none"> <li>▪ Overview of studies performed in phase I, II and III</li> <li>▪ Standard PK sampling employed in Phase I and II.</li> <li>▪ Use of sparse sampling and population PK approaches in Phase III.</li> </ul> </li> <li>○ Discuss importance of validation of analytical methods – as a regulatory requirement.</li> </ul> </li> </ul>	<b>Marco Siccardi</b> ESQlabs

Time	Activity	Speaker
10:30	<b>Tea/ coffee break</b>	
10:50	<b>Clinical Pharmacodynamics</b> <ul style="list-style-type: none"> <li>• First in human trials</li> <li>• Guideline</li> <li>• Objectives of clinical pharmacodynamic studies</li> <li>• Mechanism/onset/duration of action</li> <li>• Examples of pharmacodynamic models</li> <li>• Different study designs</li> <li>• Identification of sub-group differences e.g. disease-related, gender, age, race, geography (racial sub-populations)</li> <li>• Biomarkers</li> <li>• Practicalities of clinical pharmacodynamic studies</li> </ul>	<b>Marco Siccardi</b> ESQlabs
11:30	<b>Panel Discussion</b>	
12:00	<b>Lunch</b>	
13:00	<b>Optimal Study Design – Objectives and Issues Relating to Phase II studies</b> <ul style="list-style-type: none"> <li>• Objectives of Phase II studies</li> <li>• “Proof of concept”</li> <li>• Design of Phase II studies</li> <li>• Definition of target patient population</li> <li>• Choice of end point(s)</li> <li>• Dose response</li> <li>• Initial identification of possible safety issues</li> <li>• Importance of keeping the target product profile in mind throughout</li> <li>• Adaptive design and accelerated development</li> <li>• Conditional approval</li> </ul>	<b>Carly Barraclough</b> Amgen LTD
14:00	<b>Paediatric Investigation Plans</b> <ul style="list-style-type: none"> <li>• Legal framework</li> <li>• Why children are different</li> <li>• Preferred approaches to clinical development in children</li> <li>• Devising PIP strategy</li> <li>• Content and format of a PIP</li> <li>• PIP review process</li> <li>• Compliance Check</li> </ul>	<b>Steve Pinder</b> Envestia Ltd
14:35	<b>Case study and feedback session</b> <i>Tea to be taken in case study groups</i>	
17:00	<b>Close</b>	

## CRED: Understanding Clinical Development Programme

### Day two

**Chairperson:** Beatrix Friedeberg, Vertex Pharmaceuticals

Time	Activity	Speaker
08:55	<b>Introductory comments</b>	<b>Chair</b>
09:00	<b>Design of Clinical Trials to Support Proof of Efficacy (Phase III)</b> <ul style="list-style-type: none"> <li>• Confirmation of efficacy in the target patient population</li> <li>• Considerations for trial design e.g. control groups, duration of treatment</li> <li>• Long term safety data (circumstances when needed)</li> <li>• Choice of comparator (placebo vs active comparator)</li> <li>• Statistical issues – stats plan, primary and secondary endpoints, exploratory endpoints</li> <li>• Enlargement of the safety data-base to support the safety sections of the SmPC</li> <li>• Inclusion of quality of life (QoL) and other pharmaco-economic endpoints to support pricing/reimbursement</li> <li>• Master protocols</li> </ul>	<b>Beatrix Friedeberg</b> Vertex Pharmaceuticals
10:00	<b>Tea/ coffee break</b>	
10:30	<b>Safety</b> <ul style="list-style-type: none"> <li>• Pharmacovigilance - aims and objectives</li> <li>• Definitions</li> <li>• Clinical Trial Regulation – Reporting</li> <li>• Causality attribution</li> <li>• Risk management plans</li> <li>• PASS Studies</li> <li>• The SPC</li> <li>• Current EU Pharmacovigilance Legislation – mention Reference Safety Information (RSI) and new guidance</li> </ul>	<b>Janet Jepras</b> Janet Jepras Consulting Ltd
11:30	<b>Panel discussion</b>	
12:00	<b>Lunch</b>	
13:00	<b>Case study and feedback session</b> <i>Tea to be taken in case study groups</i>	

Time	Activity	Speaker
15:15	<b>The Perspective of a Regulatory Authority Reviewer</b> <ul style="list-style-type: none"> <li>• Specific examples of what regulatory agencies look for</li> <li>• Common problems with the clinical data in MAAs</li> <li>• Reasons for different views and decisions between regulatory authority reviewers</li> <li>• Obtaining regulatory agency input and appropriate timelines <ul style="list-style-type: none"> <li>○ CHMP scientific advice versus national agency advice</li> <li>○ Implementation of advice received</li> </ul> </li> </ul>	<b>Jana Zizkovska</b> State Institute for Drug Control (SUKL)
16:00	<b>Summary</b>	<b>Chair</b>
16:30	<b>Close</b>	

*Delegates will be encouraged to ask questions throughout the day so as to ensure the meeting is as interactive as possible.*