Managing Uncertainty over the Life Cycle of Drug Development and Use
Enhancing Adaptability and Flexibility in Pharmaceuticals Innovation

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Introduction

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Recent Developments in Europe

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Recent Developments in the United States

Dr. Anton Hoos, Director, Medicines 4 Patients

Former Senior VP European Medical Affairs & Head Global Rare Diseases GSK

Industry and Patient Perspectives on Recent Developments
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<th>CRISES</th>
<th>RESPONSES</th>
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<tr>
<td>Thalidomide</td>
<td>Approval based on efficacy/safety evidence from trials</td>
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<td>Strengthen adverse effects reporting (AERS/VARS)</td>
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<td>Accutane™ and Vioxx™</td>
<td>Controls to limit known risks (REMS/RMS)</td>
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<td>Increased attention to safety and risk management</td>
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<td>Strengthen active &amp; passive surveillance (Sentinel)</td>
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<td>HIV, Cancer</td>
<td>Accelerated Approval/Conditional Marketing Authorization</td>
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<td>Use of un-validated biomarkers</td>
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<td>Licensing backlog</td>
<td>Prescription Drug User Fee Act (PDUFA)</td>
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EVOLUTIONARY CHANGES

Genetic revolution and splintering of indications
Increasing late stage failures and rising development costs
Rising drug prices
Demand for evidence based treatment and payment
Patient demand for voice
Improving (but still flawed) electronic health records
Rising liability concerns with early patient exposure
Globalization of trials, procurement and production

LEARNING AND INNOVATION?

Health Canada
Progressive Licensing

US Food and Drug Administration
Breakthrough Product Designation
Specialized Medical Use
Patient Focused Drug Development
Pharmaceutical Quality Metrics
Formal Benefit-Risk Assessment

European Medicines Agency
Roadmap to 2015
Pharmacovigilance Legislation
Adaptive Licensing Pilots
Public access to data on trials