EXPEDITING **PATIENTS' ACCESS TO** MEDICINES:
SOLUTIONS TO SIMULTANEOUS SUBMISSIONS AND APPROVALS

WORKSHOP
30 - 31 March 2009
Surrey, UK

Workshop Synopsis

A published summary of the Proceedings of this Workshop can be found in the September issue of the journal of *Clinical Pharmacology and Therapeutics* CITATION TO COME

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Background

Major companies engaging in global development programmes are currently trying to reduce the development time in worldwide markets, ideally looking for a global development programme that will lead them to simultaneous submissions and approvals across multiple markets. Sponsors and agencies are also seeking ways to improve the efficiency and quality of clinical research, including the use of innovative trial designs, and new technologies such as biomarkers and pharmacogenomics. Participants in this 2-day Workshop discussed whether integrating these novel approaches to medicine research within a global development programme can facilitate a streamlined simultaneous submission and review process that will ultimately expedite patient access to medicines.

The Sessions

This Workshop, which consisted of 14 didactic presentations and two Syndicate discussion sessions, was convened among senior representatives of the international pharmaceutical industry, regulatory agencies and academia. Presentations explored new research paradigms and regulatory initiatives and investigated whether these will enable companies to achieve successful global simultaneous submissions, reviews and approvals.

The Syndicate groups were asked to examine two topics that need to be addressed when considering a simultaneous submission programme:

- *What are the potential barriers and solutions to simultaneous submission and approval?*
- *What are the reasons for divergent regulatory opinions on the same data, same timeframe and same dossier and what can be done to mitigate the risk of divergence?*

Observations

Barriers to simultaneous submission and approval for sponsors include company structure and decision-making frameworks that may not be coordinated well enough to allow for efficiently synchronised simultaneous submissions. Furthermore, clinical practices or regulatory guidelines that differ among submission regions may obviate or limit the use of a global data set in certain countries.

Barriers for regulatory agencies to effectively review dossiers that form part of a global simultaneous submission include differences between countries in the availability and use of technology, such as that necessary for electronic CTD submission, the ready availability of secure data transfer channels and practical consideration regarding complexities of working across distant time zones. Poorly characterised clinical populations can also have a negative impact on simultaneous submissions; that is, are differences between populations evaluated in the dossier intrinsic to genetic heterogeneity or do they result from extrinsic factors such as regional variations in medical practice, product use, or clinical trial ethics, recruitment, conduct and data analysis? Further, differences exist in the acceptance of surrogate endpoints or biomarkers across global agencies.

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Recommendations

- Determine each medicine's suitability as a candidate for a simultaneous submission; not all medicines are appropriate targets for this type of programme.
- Maintain early and open discussions with the relevant agencies regarding the plans for simultaneous submission, allowing optimal agency resource allocation.
- Seek and engage in parallel scientific advice as frequently as possible with the relevant agencies. In addition to the potentially expedited access to new medicines, simultaneous communication with multiple agencies may result in a broader scientific perspective and the opportunity to make use of specialised expertise within each country.
- Formalise a standardised benefit-risk assessment methodology (ie, framework and appropriate models) across the relevant agencies; establishing a common framework will help to identify the main factors that are barriers to achieving the common goal of reaching agreement on the benefits and risks of a new medicine, and can promote open discussion of those issues that impede a common understanding of divergences.
- Commission work to identify the intrinsic and extrinsic factors that may affect the comparability of clinical data (ie, genetic-based versus cultural-based differences), resulting in a development plan yielding globally acceptable data.
- Seek out creative means to enable data sharing and communication through novel IT solutions, thereby facilitating the most timely exchange of information between multiple agencies and among agencies and sponsors operating in different time zones.
- Survey regulatory authorities and industry on their experience with different methods of providing/obtaining simultaneous regulatory guidance from more than one regulatory authority, thereby identifying best practices for simultaneous submission and review and optimising the opportunity for predictability.
- Develop a Global Tool Box that can serve as the basis for creating risk management plans that can be deployed consistently across many regions. A common framework will ensure the monitoring and dynamic assessment of safety parameters.

Workshop Presentations

Session 1 Chair: Professor Hans-Georg Eichler, Senior I Medical Officer, EMEA

Dr Franz Pichler, CMR International Institute for Regulatory Science
Dr. Supriya Sharma, Director General, Therapeutic Products Directorate, Health Canada
Robin Evers, Vice President Head of Global Regulatory Affairs, Europe, Middle East & Africa, Wyeth Europa
Dr Paul Huckle, Senior Vice-President, Global Regulatory Affairs, GlaxoSmithKline
Tracy Baskerville, Head, Global Regulatory Affairs, Liaison, Cardio-Metabolic, Solvay Pharmaceuticals
Dr Murray Lumpkin, Deputy Commissioner International Programs, FDA

Session 2 Chair: Professor Robert Peterson, Clinical Professor of Paediatrics, University of British Columbia Faculty of Medicine

Dr Damian O'Connell, Pfizer Clinical R&D
Alison Lawton, Senior VP, Global Market Access, Genzyme Corporation
Dr Eric Abadie, Head, CHMP, EMEA
Dr Leonie Hunt, Head Office of Prescription Medicines, Therapeutic Goods Administration
Professor Trevor M. Jones, CBE, Member of the Scientific Committee, IMI
Dr Alberto Grignolo, Corporate VP, Global Strategy and Services, PAREXEL Consulting, Member, CTTI Executive Committee

Syndicate Sessions

Chairs: Dr Tomas Salmonson, CHMP Member (Vice Chairman), Medical Products Agency, Sweden; Professor Sir Alasdair Breckenridge, Chairman, Medicines and Healthcare Products Regulatory Agency (MHRA), UK
Rapporteurs: Dr Kathryn Broderick, Eli Lily Co, US; Dr Victor Raczkowski, Solvay Pharmaceuticals, US

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