Access: the key to successful European commercialisation – rising to the challenge(s)

Wills Hughes-Wilson, SVP Access & External Affairs, Chief Patient Access Officer, Sobi

wills.hughes-wilson@sobi.com
Europe: a large, diverse set of systems

- EU 28 countries: 500+ million people
- Non EU countries
- 1 EU Marketing Authorisation...
- ...28+ national, regional, local systems for
  - HTA evaluations
  - Individual Pricing & Reimbursement decisions
- Different healthcare traditions & systems
- Increasing access issues?
Increasing collaboration, impact on end-result?

CHMP
Regulatory Evaluation

PRAC
Safety & Efficacy Follow-up plans

HTA
Relative Effectiveness Evaluations
Orphan drugs face amplified challenges

- Regulatory risk:benefit – unmet medical need
- Uncertainty of evidence – strength, statistical significance
- New and emerging technologies – “taking it on trust”?
- Techniques may be part of treatment – limited centres

BUT

- Acknowledgement of shared challenges
- Systematic inclusion of stakeholders
- Willingness to collaborate to find solutions
- Opportunities at every step of the way
- Charting a path
Right dialogue at the right time: Where we are will determine what could be useful

- Innovation Task Force
- Business Pipeline Review
- Adaptive Pathways
- The future...?
"This is not so much an adaptive system as an adaptive mind-set"  

"It’s a prospective plan, agreed with all stakeholders, to progressively reduce uncertainty + grant access to needed therapies"
Where we are will determine what could be useful
Parallel Regulatory + HTA Scientific Advice

- Individual countries
- European Medicines Agency (EMA) “Parallel” Scientific Advice
  - Since 2010
  - 25+ procedures already by 2013
  - Well-established opportunity – guidance, best-practice
- SEED – Shaping European Early Dialogues for Health Technologies
  - 14 HTA bodies, coordinated by HAS, France
  - Funded by EU: October 2013-August 2015
  - 10 early dialogue projects – 7 medicines, 3 medical devices
  - “Reduce the risk”
Where we are will determine what could be useful

Phase 1 > Phase 2 > Phase 3 > Follow-up > HTA Health Technology Assessment > Pricing & Reimbursement
Aligning on Health Technology Assessments (HTAs)

- Since 2005 collaboration – 30 countries in “Joint Action 2”
- Co-funded EU and HTA bodies
- Collaboration for evidence-based decision-making
- Reduce duplication, share knowledge
  - Efficient use of resources
  - Sustainable system of knowledge-sharing
  - Best practices
- 10 pilots, “production” – an interest in orphans
- Uptake and use by Member States
- Participation in the development of the future systems, tools
Where we are will determine what could be useful

Phase 1
Phase 2
Phase 3
Follow-up
HTA Health Technology Assessment
Pricing & Reimbursement
Follow-up measures – safety + efficacy

- Pragmatic regulatory decisions since earliest days of Orphan Regulatory framework
- Uncertainties at time of Marketing Authorisation – follow-up
- Conditional Marketing Authorisation since 2006
- EU’s Pharmacovigilance Regulatory framework 2012 & 2014: Pharmacovigilance & Risk Assessment Committee (PRAC)
  - Post-Authorisation Safety Studies (PASS)
  - Post-Authorisation Efficacy Studies (PAES)
Where we are will determine what could be useful
Multiple opportunities to interact with payer bodies

- Talking with individual countries
  - Planning, budgeting
  - “No surprises” – for anyone!

- MEDEV group of payers exploring MoCA recommendations
  - Set of recommendations about joint evaluation of orphan drugs signed by 11 EU Member States, April 2013
  - Payers “club” since 1998, now inviting orphan drug manufacturers for dialogue on a voluntary basis
  - 1 pilot initiated, 2 further pilots initiated

- Monday 20 April 2015 – Belgium + Netherlands formally announce intent to collaborate on orphan pricing negotiations
  - Develop practical action points to enable joint discussions with companies
  - Intent to be operational by 2016
  - “Represent more patients, can negotiate a lower price”
But if Europe comes late in the thinking, what then?
Small number of specialised companies

Ex-EU company

- 1 niche product
- +/- 300 patients in EU
- Ph3 or later
- No EU capabilities

- Costs/capital
- Regulatory
- Pricing & Patient Access
- Pharmacovigilance
- Compliance
- Supply network
- Marketing capabilities
- QA release
- KOL network
- IT
- Insurances
- Offices
- ...

Estimated start-up cost in EU $50 million!
A wealth of initiatives to address uncertainties, build collaboration
Whose responsibility is it?

Creating a prospective, agreed system

- Horizon Scanning
- Payers
- Early Dialogue
- Post-Authorisation Safety & Efficacy
- Real-World Patient Outcomes
- Conditional Reimbursement
- Conditional Value-Based Pricing
- Conditional Marketing Authorisation
- Initial Value Assessment
If we have a shared goal, we can find solutions
Rising to the challenge(s)

• It should be part of your first move
  • Have the end in mind from the very beginning – securing [reimbursed] access will be a critical key to commercialisation + avoiding delays

• The innovative product is just the start – need similar innovation in your team approaching the European market(s)
  • Am I making the most of everything at my disposal?
  • Engagement, adaptive designs + methods, discussing at European / collaborative levels

• It’s never too late to start a dialogue – with whom will depend on where you are in the process + it’s [almost] never too late

• People across the system are genuinely open to find collaborative solutions if you are genuinely seeking the same outcome
Thank you!