Response to NHS Chief Executive’s Open Call for Evidence and Ideas

Respondent ID: 195

Organisation name: Shire Pharmaceuticals Ltd

Type of response: Document
Contact details of you and your organisation
We would like to be able to follow up interesting comments and case studies and would be grateful if you can give your contact details below.

**Organisation:** __________Shire Pharmaceuticals Ltd____________________________

**Contact (completed by):** __Paul Cox____________________________

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Do you want to be kept in touch with the next steps in this process? **Yes**

Do you want to be included in a wider community of interest? **Yes**

Information about your organisation
If you are responding on behalf of an organisation, please could you indicate which best describes the role of your organisation.

**Name of your organisation:** __Shire Pharmaceuticals Ltd____________________________

Please choose the description below that best fits your organisation's main role:

1. NHS Trust / NHS Foundation Trust
2. PCT / clinical commissioners
3. SHA
4. General Practice / Community services
5. NHS other
6. Social care sector
7. Independent healthcare sector
8. Royal College
9. National / Local Government
10. Voluntary/charitable sector

**11. Private sector (including life sciences sector)**

12. Academic Institutions
13. Representative body
14. Public/Patient
15. Other
Shire plc is a FTSE100 company. We are a leading company in the development and commercialisation of therapeutics for the patients suffering from rare diseases around the world. Almost 50% of our employees are engaged in researching, developing and making available therapies for these patients with significant unmet medical needs.

**General points**

- Shire welcomes the commitment made by the NHS to support innovation.

- We believe that the review represents an opportunity to bring about actions within the NHS that have a material impact on the uptake of innovative medicines for the benefit of patients.

- However, we believe that the review must produce real outcomes that make a difference and ensure that frontline healthcare professionals are able to achieve tangible access to recognised and agreed innovations (including new therapeutic interventions) for the benefit of their patients.

- We are concerned that, with various ongoing initiatives, there must be a consistent, clear definition of what is meant by innovation so that there is universal recognition of innovative medicines that leads directly to access for patients and is not subject to further interpretation of the benefits or their value.

**Case study - Firazyr (icatibant)**

- Firazyr was developed by Shire and is used in treating acute attacks of hereditary angioedema (HAE) in adults 18 years of age and older.

- Firazyr was recognized as an innovative medicine in 2010 winning the Prix Galien, Orphan Drug category. The award being presented by Secretary of State for Health Rt Hon Andrew Lansley CBE MP based on the findings of the judging panel Chaired by Professor Sir Michael Rawlins [see below for further background]

- However, it has taken 2 years to gain even limited access to Firazyr by experienced Immunologists for their HAE patients. As a new medicine, not part of the NICE workplan, Firazyr has been subject to continued additional reviews at local level (PCT or consortia thereof), individual patient requests and additional formulary review which has added significant delays to access by patients to this innovation.

- We believe Firazyr is a relevant example of the difficulties currently experienced in the NHS by healthcare professionals and their patients in gaining access to recognised innovations and would welcome involvement in any further involvement of industry in this consultation.

**Actions at national level**
Shire believes that the NHS Commissioning Board must apply a consistent definition of innovation and must ensure that it is applied in a consistent manner operationally within the NHS.

This is particularly important for innovation that is associated with rare conditions, where we believe that services and innovative medicines must be closely linked and both must be commissioned at national level. We are not convinced that local commissioning groups will have the breadth of perspective effectively to commission innovative medicines for rare diseases; focusing, as they will, on local priorities.

Where formalised decision making processes are used, these should incorporate consideration of the innovative nature of a particular medicine.

Shire supports the calls for an innovation fund to speed up access to new innovative medicines, services etc.

**Actions at local level**

We believe that decisions on innovative medicines to treat rare conditions should take place at national level. There is therefore little action that would be appropriate from local NHS organisations with the following exceptions:

- The early identification of patients who might benefit from treatment
- Provision and maintenance of appropriate non-specialised services to support patients using innovative new treatments

**NHS partners**

Shire supports the calls for registries to be put in place to aid patient identification

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Background to Prix Galien awards

http://www.prixgalien.co.uk

Extract from Press Release on 2010 Prix Galien Awards 14th Oct 2010

UK Prix Galien 2010

Promoting innovation is a central aim of Prix Galien, as outlined by Professor Sir Michael Rawlins, Chairman of NICE and head of UK Prix Galien's judging panel, who announced the winners. “Prix Galien is about honouring excellence in pharmaceutical research and development,” said Professor Sir Michael. “It is about recognising the contribution that new medicines can make to the lives of people with life-threatening conditions. It is about celebrating the achievements of all those individuals – working as teams – upon whom we rely for the discovery and development of new medicines. Most will be unknown to us – but we all owe them a huge debt of gratitude.”

The 2010 Prix Galien comprised a total of 10 shortlisted products – 6 for the Innovative Product Award and 4 in the Orphan Drug category. Professor Sir Michael said that the judging panel had been “immensely impressed” by the creativity, innovation and scientific rigour that have gone into the discovery and development of all the shortlisted medicines. “In their own way, each offers hope to thousands of people and their families,” he added.

Orphan Drug Award

The Orphan Drug Award was introduced as a dedicated category at the previous UK Prix Galien in 2008, following a special award for orphan products in 2006. The term ‘orphan condition’ is used to describe conditions that affect a very small number of patients in a given population - many of which are either untreatable or treated very inadequately. It is estimated that there are 6000 orphan diseases – which, in total, affect about 30 million EU citizens.

Parliamentary Sponsor, Lord Walton of Detchant – a Crossbench Life Peer and former Prix Galien judge – said the decision to give a new award for orphan drug discovery was “farsighted” and commended the industry on its developments in this important area of medicine. “In many branches of medicine, there are many diseases formerly unresponsive to any pharmaceutical intervention which are now known to be due to single gene disorders. Many of these are rare, and as drugs begin to emerge capable of circumventing the genetic defect, the costs of development may be huge, but because of the rarity of the relevant conditions, the income derived from marketing such remedies may be relatively limited,” he said. “This is a huge dilemma for the industry to face. It is good to know that there have been several outstanding submissions in the orphan drug category this year.”

Professor Sir Michael Rawlins concurred. “For orphan diseases that are potentially treatable with medicines, pharmaceutical manufacturers face a number of hurdles, including concerns about the size of the market and difficulties – because of the small numbers of patients – in their development. The judging panel was delighted by how many companies are now working in these areas – and with the extremely high calibre of the entries to this category.”

Winner: Firazyr® (Shire Pharmaceuticals)

The Orphan Drug Award 2010 was won by Shire Pharmaceuticals for its hereditary angioedema (HAE) treatment, Firazyr. HAE is a rare condition characterised by recurrent episodes of oedema formation in the soft tissues of the extremities, face, genitals as well as in the mucous membranes of the gastrointestinal tract and larynx. These attacks are often painful, disfiguring and debilitating. And involvement of the larynx can be lethal.

The exact prevalence of hereditary angioedema is uncertain – but estimates suggest that 1:10,000 to 1:50,000 persons are affected. Hereditary angioedema is an autosomal dominant
disease caused by the absence or dysfunction of C1-esterase inhibitor. While the exact trigger for attacks of hereditary angioedema is unknown, it follows activation of the complement cascade with increased local production of bradykinin. Firazyr selectively – and competitively – inhibits the action of bradykinin – thus halting oedema formation and rapidly alleviating symptoms. In clinical trials, Firazyr significantly reduced the duration of HAE attacks. In 90% of patients only one injection was required to achieve effective control of the HAE attack.

“Apart from its novel mechanism of action and clear evidence of its clinical effectiveness, the jury were also extremely impressed by the clinical development programme of Firazyr,” said Professor Sir Michael. “Attacks of hereditary angioedema occur spontaneously and without warning. Patients tend to seek help from their nearest Accident and Emergency Department or Emergency Room. Organising clinical trials under such circumstances is what might be called ‘a challenge’! That Shire managed to undertake the clinical development of this product in such an unpromising environment – is hugely to their credit.”

“We are delighted to see that by driving innovation in orphan diseases our efforts have been rewarded’ said Janis Clayton, VP & General Manager Shire UK & Ireland “This prestigious recognition highlights the outstanding work undertaken by the UK and global teams who have given patients with Hereditary Angioedema an innovative treatment for their condition. Shire will strive to remain at the forefront of orphan medicine.”

“This is a huge accolade for the pioneering research that has been not only challenging, but hugely rewarding” said Dr Aidan Gill, Medical Director, UK & Ireland. “We are honoured that our work with HAE has been recognised by this reputable and stringent awards board. It only serves to show that the united approach of the Shire HAE team has proven to be highly successful. We will continue to work in partnership with the clinical community to have a positive impact on this and other debilitating diseases.”

**Summary**

Demonstrating the value of medicines remains a key priority for the pharmaceutical industry as it battles turbulent global economic conditions. The Prix Galien serves as a powerful reminder of the value pharmaceuticals play not only in the ongoing challenge to improve patient care, but also in the national economy. “In these difficult times, when we face substantial reductions in public expenditure, it is vital to this country’s industrial future that research and development should be protected,” said Lord Walton. “The quality of the entries submitted for the two awards this year has clearly been exceptional. May the industry continue to prosper, and may many innovative and exciting remedies be submitted for consideration next time, while also being made readily available to the great benefit of our patients. And may our new Government also be persuaded of the inestimable value of the industry and of its products.”

In response, Andrew Lansley said: “Our objective is to build an NHS that is going to assess outcomes. If the pharmaceutical industry can deliver innovation – and these Prix Galien awards have repeatedly demonstrated that it can – we will all see the reward that comes from it. You will have not only the reward in the remunerative sense, but also the reward of knowing that you are contributing to the dramatic increases that we can all achieve in outcomes for patients, results for patients, healthy life expectancies in this country and combating the many burdens of disease that we’re all fighting against.”

**NOTES FOR EDITORS**

1. Prix Galien is also awarded in 11 other countries; Belgium, Canada, France, Germany, Holland, Italy, Luxemburg, Portugal, Spain, Switzerland and the US. An International Award is also held every two years.

2. The Prix Galien was created in France by pharmacist Roland Mehl, whose aim was to promote the country’s significant advances in pharmaceutical research. Until the establishment of the Prix Galien, this discipline remained unrecognized. Mehl brought together an eminent jury consisting of clinicians, toxicologists, pharmacologists and pharmacists to select and honor the most important drugs introduced to the public market and the most significant research teams in the pharmaceutical field. Since then, Prix Galien has been introduced across Europe and Canada and is now the most prestigious award of its kind in 11 countries. In addition, an International Prix Galien award is given each year, selected from previous winners.
3. The UK Prix Galien judges are;

**Chairman:** Professor Sir Michael Rawlins; Professor of Clinical Pharmacology, University of Newcastle upon Tyne. Chairman, National Institute of Health and Clinical Excellence (NICE).

**Vice-Chairman:** Professor Parveen Kumar; Professor of Medicine and Education at Barts & The London School of Medicine and Dentistry, Queen Mary, University of London and Honorary Consultant Physician and Gastroenterologist at Barts & The London NHS Trust, Homerton University Hospital Foundation Trust.

**Professor David Barnett:** Professor David Barnett; Emeritus Professor of Clinical Pharmacology at the University of Leicester. Former Professor of Clinical Pharmacology at the University of Leicester and Honorary Consultant Physician with a special interest in cardiovascular medicine at the University Hospitals of Leicester NHS Trust.

**Professor Dame Carol Black:** National Director for Health and Work, Chair of the Nuffield Trust and Chair of the Academy of Medical Royal Colleges, immediate past President of the Royal College of Physicians.

**Professor Sir Gordon W Duff:** Florey Professor of Molecular Medicine, University of Sheffield, and Honorary Consultant Physician in the Sheffield Teaching Hospitals NHS Foundation Trust.

**Professor Deirdre Kelly:** Professor of Paediatric Hepatology, Birmingham Children’s Hospital

**Dr Mark Porter:** General Practitioner and Media Doctor

**Professor Sir Mike Richards:** National Clinical Director for Cancer and End of Life Care

**Professor David J Webb:** Christison Professor of Therapeutics and Clinical Pharmacology, University of Edinburgh, Chairman of the Scottish Medicines Consortium (SMC)