

# Consultation Response

## Innovation Pass Pilot

### Department of Health



Response by the Genetic Interest Group and Rare Disease UK

The Genetic Interest Group (GIG) is the UK national alliance for all people affected by genetic conditions. GIG works to raise awareness and improve the health service provision available to those living with and at risk from inherited conditions. Our membership represents 138 voluntary organisations working for a wide range of conditions, many of which are rare and/or pose complex health and social care needs.

Rare Disease UK (RDUK) is the National Alliance for people with rare diseases and all who support them. It is a joint initiative of the Genetic Interest Group and other key stakeholders including over 100 patient organisations, clinicians, academics, industry and interested individuals brought together in response to the unmet care needs of the 3.5 million people who struggle to access integrated care and support from the NHS. Approximately 85% of rare conditions are genetic.

Many of the organisations, patients and families that GIG and RDUK represent are affected by conditions for which there is no cure or treatment currently available. As a result, we believe that it is vitally important that the development of new therapies, medical technologies and diagnostics are encouraged in order to make a real improvement to the lives of patients and families living with rare and genetic disorders and to ultimately find a cure for these conditions.

GIG and RDUK welcome the opportunity to respond to this consultation.

## Introduction

The Office of Life Sciences Blueprint published in July 2009 recognised the unique potential the NHS has to lead the way in the uptake of innovative medicine and touted the NHS as “an innovation champion”. This also ties in with the NHS’s focus on Quality, Innovation, Productivity and Prevention (QIPP). We were pleased with the trend towards encouraging innovation and addressing some of the obstacles to the development and uptake of innovative therapies that currently exist.

GIG and RDUK are particularly interested in overcoming the impediments to the development of innovative treatments for rare conditions. Companies developing these treatments experience additional difficulties as compared with those developing conventional drugs; for example, a return on investment with such small patient populations is unlikely without specific additional incentives. Constraints are not solely financial however. Working with small patient populations can make the practical development of treatments more difficult. Recruitment for clinical trials can be a lengthy, difficult process; and it may be

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impossible to achieve the patient numbers required for results of trials to be considered statistically significant under conventional health technology assessment rules.

By proposing an “Innovation Pass”, the Office of Life Science sought to address one of these obstacles. It recognised that there are drugs aimed at small patient populations where there simply isn’t enough data to satisfy NICE’s cost-effectiveness tests, often due to the difficulties involved in collecting data on clinical effectiveness when small numbers of patients are involved.

We are pleased with the recognition of the issue and with movements towards addressing the problem. In the circumstances outlined, we are concerned that NICE presents a major obstacle which further discourages the development of therapies for many of the conditions represented by RDUK and by GIG where there are small patient numbers.

However, whilst we welcome any scheme put in place aimed at facilitating the access to new potentially life enhancing therapies and consequently encouraging the development of new innovative products, GIG and RDUK are disappointed with the scope and ambitions of the Innovation Pass pilot presented in this consultation. Below we outline some of our main areas of concern.

## Narrow scope

We were disappointed to find that in practice the scope of the Innovation Pass had been limited to effectively exclude drugs for people with very rare conditions. The consultation states:

“The Innovation Pass is not likely to constitute an appropriate means of dealing with drugs for extremely small patient populations where the economics of an intervention are unlikely to improve over time e.g. where there is little or no prospect of significantly better evidence, the patient population is so small and the nature of the clinical indication so specific that the drug is priced at a level that could never achieve a positive NICE appraisal.”

Whilst it is not specified what constitutes an “extremely small patient population” it seems likely that the Innovation Pass would exclude what are known as “ultra-orphan drugs”, i.e. drugs to treat conditions with a prevalence of less than 1 in 50,000 persons. Furthermore, it could potentially exclude many orphan drugs for conditions affecting no more than 5 in 10,000 persons.

Drugs for “ultra orphan” and orphan conditions can be among the most innovative of products; for example, the first enzyme replacement therapy for Gaucher’s Disease delivered a paradigm shift in treatments for inborn errors of metabolism. By narrowing the scope of the Innovation Pass to exclude treatments for very rare conditions we believe an opportunity has been missed, not only to encourage the development of new drugs for rare diseases, but also to stimulate research and to gain a better understanding of the route of action of novel drugs; the results of which could have knock on benefits for more common conditions.

In the Blueprint, the Innovation Pass was initially proposed for small patient populations in order to “give earlier access to innovative drugs for patients with the greatest need”. By effectively ruling out its use for very rare conditions the Innovation Pass Pilot provides no solution for patients with the greatest need.

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## A lack of incentive

Development of new therapies for rare diseases has been long neglected due to the lack of perceived opportunity/cost by the pharmaceutical industry; as a result it is widely accepted that incentives are needed to encourage the pharmaceutical industry to develop and market therapies for rare diseases. An Innovation Pass had the potential to incentivise the development of medicines for rare diseases by assisting the collection of data to demonstrate its effectiveness.

The Innovation Pass pilot may give earlier access to a very limited number of innovative drugs for small patient populations. We are concerned, however, that the Innovation Pass pilot as proposed will have little impact on the ultimate aim of providing additional incentives to companies to invest in research and development for innovative drugs especially for rare diseases.

Due to the small market to recoup the significant research and development costs, innovative orphan drugs are often expensive with incremental cost effectiveness ratios (ICERs) at the high end or beyond what NICE considers to be cost effective using the ICER/QALY (quality adjusted life year) approach. The collection of data to demonstrate the effectiveness of an orphan drug can also be problematic due to the difficulty in recruiting patients for clinical trials and the difficulty in gathering enough evidence to satisfy standard health technology assessment processes.

The NICE appraisal process is not problematic for all orphan drugs, but we are concerned that the criteria often prove an obstacle and that this disincentive will outweigh any incentive offered by the Innovation Pass. The Innovation Pass may facilitate the collection of more data to enable a drug to demonstrate cost effectiveness, yet the fact remains that many innovative orphan drugs are likely to be expensive and companies are less likely to invest in them when they are subject to the same appraisal process as non-orphan drugs. Although the appraisal process itself is outside of the scope of the consultation we are concerned that the Innovation Pass does little to address one of the main impediments to the development of innovative orphan drugs.

## Conclusion

We welcome the movement towards fostering and encouraging the development of innovative products and to developing a framework to enable the gathering of data on the effectiveness of these products. This is likely to become an issue of increasing importance in future as we move away from “blockbuster” drugs towards targeted therapy for smaller population groups which will necessitate new frameworks for determining clinical effectiveness. The experience gained through the Innovation Pass pilot should be used to aid the development of such frameworks.

Whilst we recognise that it is a pilot scheme, GIG and RDUK are disappointed with the proposed Innovation Pass. The narrow scope and the lack of incentive that the Pass offers will not provide a solution to the problem of access to innovative medicine for small patient populations. The Innovation Pass pilot may facilitate speedier access to some innovative drugs but we are concerned that this will apply only to drugs for a very small range of conditions (especially given the budget that has been

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allocated). As the pilot effectively excludes drugs for patients with very rare conditions it also rules out some of the patients with the greatest need.

It is our view that the pilot will do little to encourage the uptake of innovative medicine to meet the aim of the “NHS as an innovation champion” as envisioned by the Blueprint. Nor will it provide enough of an incentive to encourage the development of innovative therapies.

## Recommendations and future review of the Innovation Pass

- The Department of Health and NICE need to work together to develop a new system for appraising therapies for orphan and “ultra-orphan” diseases. The particular difficulties involved in developing these therapies and the small patient numbers involved mean that access should not be determined solely by a cost-effectiveness exercise.
- We urge the Department of Health to consult on developing further the processes for evaluating interventions for rare conditions as part of national specialised commissioning arrangements (as stated in para.3.1 of the consultation document).
- The issue of how to incentivise the development and aid the uptake of innovative medicine for rare and very rare diseases should be explored.
- Should the Innovation Pass Pilot prove to be effective its use should be rolled out further to include other interventions (Question 1).
- Consideration should be given to how to gather evidence on the effectiveness of non-drug innovations e.g. gene transfer, stem cells, and tissue engineering, given their added complexity.
- There should be collaboration with regulators such as the European Medicines Agency and all relevant stakeholders to look into new frameworks for assessing the cost and clinical effectiveness of innovative medicine, including the final Innovation Pass once it has undergone its pilot stage. Collaborative working should be embraced also to learn from experience in the US and Europe.
- Non-financial incentives should be considered to aid the development and uptake of innovative medicine e.g. extra support could be to be given to small and start-up enterprises developing innovative products in order to aid their understanding of the demands of the assessment process and the collection of data on the effectiveness of their product. An example of this can be found at the European Medicines Agency; the Committee for Medicinal Products for Human use (CMPH) established the Scientific Advice Working Party (SAWP) with the aim of providing Scientific Advice and Protocol Assistance for orphan medicinal products.



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