Progress Update - Access to newly licensed medicines

Genetic Alliance UK

Introduction

1. Genetic Alliance UK is the national charity working to improve the lives of patients and families affected by all types of genetic conditions. We are an alliance of over 180 patient organisations. Our aim is to ensure that high quality services, information and support are provided to all who need them. We actively support research and innovation across the field of genetic medicine.

2. Rare Disease UK (RDUK) is a multi-stakeholder campaign run by Genetic Alliance UK, working towards the delivery and implementation of the UK Strategy for Rare Diseases\(^1\), signed by all four health departments in the UK and published by the Department of Health in November 2013.

3. Genetic Alliance UK thank the Health and Sport Committee for conducting a review of the reforms carried out by the Scottish Medicines Consortium(SMC) and Scottish Government relating to access to new medicines and welcome the opportunity to provide written evidence.

4. In 2011, Rare Disease UK (RDUK) submitted Public Petition PE1398, calling for a thorough review of the processes used to assess new medicines in Scotland. Rare Disease UK is the national alliance for people with rare diseases and all who support them, it was established by Genetic Alliance UK.

5. Public Petition PE1398, and Public Petitions PE1399 and PE1401 submitted by AGSD UK, PNH Alliance and PNH Scotland, called for a review of the way orphan and ultra-orphan medicines are assessed by the Scottish Medicines Consortium and for a thorough review of the Individual Patient Treatment Request (IPTR) process. We welcomed the findings and recommendations of the 2013 Health and Sport Committee Inquiry and those of the Scottish Government Review on Access to New Medicines in Scotland carried out by Professor Routledge and Professor Swainson.

6. Genetic Alliance UK are currently undertaking a project to develop a Patient Charter on Access to New Medicines for Rare Diseases in Scotland. In October 2015, we held a consultation event with patient organisations to review the processes for accessing new medicines in Scotland. The Patient Charter will be finalised and launched in Spring 2016, however the findings from our consultation event have informed our response to this call for evidence.

7. It is the opinion of Genetic Alliance UK that the reforms that have been carried out as a result of the 2013 reviews have resulted in an improved system for accessing new medicines for rare conditions, however there are still improvements that can and should be made. Below, we have highlighted the key successes, issues arising and our recommendations for further improvement. We look forward to providing further evidence at the Health and Sport Committee evidence session on Tuesday 9th February 2016.

Scottish Medicines Consortium

8. Genetic Alliance UK would like to thank the Scottish Medicines Consortium for the manner in which it has embraced change and implemented recommendations following the Scottish Government and Health and Sport Committee reviews in 2013. SMC have taken significant steps to improve transparency and understanding of the SMC and the introduction of the Public Involvement Team has been most welcome, providing an opportunity for meaningful patient involvement in the SMC processes. We note that there has been a positive trend in SMC decisions for rare conditions, however it is not yet possible to ascertain whether this trend is a result of changes made to the appraisal process for orphan, ultra-orphan and end of life conditions.

Transparency of SMC Processes

9. Genetic Alliance UK welcome the SMC’s commitment to improve openness and transparency of its systems and processes. The introduction of open meetings has been a valuable step forward in raising awareness and understanding of how evidence is assessed and interpreted by SMC. From the perspective of patient organisations, open meetings provide an opportunity to witness the way in which their contributions, through the Patient Interest Group Submission and Patient and Clinician Engagement (PACE) meeting, are represented.

Patient Involvement in SMC processes and decision making

10. The development of the Public Involvement Team has been a success of the recent reform. The Public Involvement Team provide a valuable source of support to patient organisations participating in both the SMC and processes. The Public Involvement Team have developed written resources, reviewed patient submission forms and undertaken a number of patient group engagement activities to raise awareness of the SMC, its role and the value of patient involvement.

11. The Public Involvement Team recognise potential difficulties in engaging with patient organisations for rare diseases, for example, patient organisations may not exist for a particular condition or may not have experience of HTA processes. The Public Involvement Team have worked with Genetic Alliance UK to identify appropriate patient organisations to participate in PACE and to discuss methods for improved patient organisation engagement for very rare condition areas.
12. Genetic Alliance UK provide a nominated individual to represent rare diseases on the Public Involvement Network (PIN). PIN comprises representatives of patient and carer groups, nominated by umbrella bodies, to ensure that the views of patients, carers and members of the public are used to inform SMC processes and to promote ongoing reform and improvement in patient involvement at SMC. The introduction of PIN has been a welcome development and an important step in improving patient involvement in SMC processes.

13. However, there is a need for an increase in the number and type of patient voice on all decision making panels at SMC. At present, SMC public partners (members of the public, working as part of the SMC Public Involvement Team) present the patient input at SMC, and patients do not have the opportunity to represent at SMC other than through the Patient Interest Group Submission or the Patient and Clinician Engagement process. It is the role of the Public Partners prepare a presentation of patient group submissions to accurately highlight key issues and messages to present at monthly SMC committee meetings. Public Partners also play a crucial role in the PACE process, participating in PACE meetings to ensure that patient and carer perspectives are fully explored and discussed and then presenting this information at the SMC meeting.

14. Genetic Alliance UK fully respect and support the role of Public Partners. However, we encourage the inclusion of patient perspectives in all decision-making processes in as unfiltered a form as possible. Patients provide an important and unique perspective in decision making, and this input is most valuable when provided in person. While we acknowledge that the current process of public partners reading PACE statements in the patient’s own words is valuable, it would be better for patients to deliver these statements directly rather than via an intermediary. Genetic Alliance UK recognise that there is value to having a non-expert public perspective at SMC, however we feel it would be more appropriate for Public Partners to represent a broader social perspective.

Recommendation 1: The role of public partner at SMC should be reviewed and consideration given to increasing the opportunities for patients to provide their perspective in person.

Information and Training for Patient Groups

15. The Public Involvement Team has undertaken excellent work to improve the quality of patient group submissions, including developing new submission forms and guidance for providing a Patient Interest Group submission and for participating in PACE. However, further comprehensive training would be welcomed to ensure patient submissions are of the highest quality. Regular training days should be undertaken for both patient representatives and clinicians to not only provide training on how to engage with the appraisal process, but also on the technical aspects of Health Technology Assessment.
Recommendation 2: SMC Public and Patient Involvement Team should hold regular training days.

16. The SMC are currently expanding a ‘PACE mentors’ programme to encourage organisations with experience of the SMC process to support other organisations to strengthen their submissions. Genetic Alliance UK support this programme and suggest further steps could be taken. Resources should be developed to share best practice examples with patient groups about to take part in the SMC process. Suggestions include developing a repository of patient group submissions or producing informative video to share examples of best practice.

Recommendation 3: SMC should consider how to share best practice relating to patient involvement in the SMC process.

17. Patient involvement in SMC decision making could be further enhanced by affording patient representatives membership of the NDC and SMC and voting rights, in a similar role to that currently held by pharmaceutical industry representatives. Patients’ experiences and preferences should be represented in all the processes which lead to the availability of new medicines, this would ensure that the benefits which really matter to patients, and the levels of risk they are prepared to tolerate are considered in the decision making process. This is particularly important for serious and rare conditions, where the stakes are so high.

18. Patient representatives (such as patient group members) should be supported as joint decision makers, alongside clinical experts, throughout the process. Similarly, to how industry gets two voting members on the SMC through their industry body (ABPI), it would be appropriate for two patient representatives to also be members provided that they are suitably trained. A trained and disinterested patient can use their insight into the potential beneficiaries’ point of view to make decisions as an active member of any body. Additionally, it would be appropriate for two patients who have made submissions to the SMC on a specific medicine to attend the SMC meeting, similarly to how submitting companies do now, in order to answer any specific questions that the panel may have and to address any issues which may require clarification.

Recommendation 4: Membership of the NDC and SMC should include suitably trained patient representatives with full voting privileges.

Recommendation 5: Patient representatives that have participated in the PACE process for a specific medicine should be invited to attend the SMC meeting to answer any questions raised by SMC members.

Clinician Involvement in SMC processes and decision making

19. Expert clinical opinion is a vital component in the SMC decision making process. While the NDC, SMC and local ADTCs may have a range of different specialisms among their membership, this does not always equate to expertise in the condition under consideration. It is essential,
particularly in the case of rare conditions, that the appropriate expert clinician be involved in decision making. It must also be recognised, that for many rare conditions, such expertise will lie outwith Scotland. Where necessary, SMC must look to the rest of the UK, or Europe, to ensure that decisions are made on the basis of all necessary information and expertise.

Recommendation 6: SMC must ensure appropriate expert clinicians are involved in decision making.

20. Expert clinicians that are invited to participate in PACE must also be experts in the disease area in question and, likewise, this may require seeking opinion outwith Scotland. For those expert clinicians that are involved in PACE, an invitation to attend the SMC meeting, whether that be in person or by teleconference, to provide answers to any questions the panel may have or to provide clarification on their PACE submission should be extended.

Recommendation 7: Expert clinicians involved in PACE should be invited to attend the SMC meeting to provide clarification or answer questions.

Transparency of SMC decision making

21. In Public Petition PE1398, it was acknowledged that there must be a robust system in place for assessing new medicines in Scotland and whilst we welcome steps to improve the appraisal process for rare diseases and end of life medicines, we remain considered about the weight of the Cost per QALY in SMC decision making. We recognise that QALYs can provide a useful indicator of an individual’s anticipated health gain following a medical intervention. However, we have concerns about how QALYs are calculated, and the weight they carry in UK health technology appraisals, including at the SMC. The ‘health related quality of life’ that QALYs measure encompass five areas of wellbeing (mobility, self-care, usual activities, pain/discomfort and anxiety/depression), with up to five degrees of measurement (from no difficulties to extreme difficulties). Patients with rare conditions agree that this method is crude and fails to capture the type and range of symptoms, emotions and disadvantages experienced by patients, families and carers affected by rare conditions. In other words, differences that may be important clinically or to the patient may not be shown by cost per QALY estimates.

22. Whilst the introduction of PACE has certainly strengthened the patient voice in SMC decision making, it is our experience (from attending SMC open meetings) that consideration of cost remains a considerable part of SMC discussions when assessing orphan and ultra-orphan medicines.

Recommendation 8: SMC should evaluate the significance of the QALY in SMC decisions for rare disease medicines.
Recommendation 9: SMC should consider applying greater flexibility when assessing rare medicines and consider removing the QALY from its central role in decision making for rare medicines.

23. Whilst the introduction of the SMC PACE process has resulted in increased patient involvement and greater patient voice in SMC decision making, it is unclear exactly what effect PACE statements have had on SMC decision making. Genetic Alliance UK members report that they feel, particularly with regards to a very high cost medicine, nothing that they could have said would have resulted in a positive decision.

24. In certain cases where an orphan or ultra-orphan medicine has been assessed and the PACE statement has been overwhelmingly supportive of its introduction, deliberations over the QALY have taken place and the medicine has been rejected. This would imply that the QALY remains the dominant factor in SMC decision making.

25. However, to date there has been no evaluation of how SMC members reach decisions and as such, it is difficult to assess what impact PACE has on the decision making process. Consultation with SMC members and research into how SMC members make decisions is necessary to truly evaluate the impact and effectiveness of PACE.

Recommendation 10: – Research to understand and monitor the impact of PACE statement on decision making should be undertaken.

Individual Patient Treatment Requests and Peer Approved Clinical System

26. Prior to the Health and Sport Committee Inquiry in 2013, rare disease patients faced significant challenges in accessing appropriate medicines through the Individual Patient Treatment Request process. This was due to a number of factors, primarily the restrictive ‘exceptionality’ criteria that had to be met and inequity in decision making across NHS Scotland Health Boards.

27. Genetic Alliance UK welcomed the interim IPTR arrangements which saw the removal of exceptionality criteria and called for a consistent and flexible approach across all Health Boards. The proposed abolition of the IPTR process and the introduction of a Peer Approved Clinical Process (PACS) which put clinical opinion at the centre of decision making was also welcomed. This new system was intended to focus on patient outcomes, and have a reduced reliance on individual requests for medicines.

Interim IPTR Guidance

28. The Scottish Government issued temporary guidance for IPTRs, SGHD/CMO (2013)20, which re-emphasised that the concept of exceptionality should not be a factor in any IPTR under consideration in local boards but should be primarily about the individual clinical case.
29. Genetic Alliance UK members have told us that they are finding the interim phase an improvement on previous IPTR arrangements, as it features a combination of added funding (in the form of the New Medicines Fund) and added leniency (the removal, in principle at least, of the exceptionality requirement) which they are finding is slightly increasing patient access via this route.

30. Whilst reliance of exceptionality has decreased and access to medicines through IPTRs seems to have improved, patient representatives have told us that even 18 months after the interim IPTR guidelines were published, a number of health boards were still using either guidance or forms that referred for the need for applicants to be exceptional. It may be due to health boards also expecting the imminent arrival of PACS, and so opting not to make significant changes to their documentation for what was anticipated to be a very short interim period.

31. Genetic Alliance UK welcome the interim arrangements for IPTRs are a pleased that this has resulted in improved access to medicines through this route. However, the interim guidance is no substitute for an improved process which centres around clinical opinion.

Recommendation 11: The interim arrangements for IPTRs must be monitored to ensure exceptionality is not a factor in decision making.

Recommendation 12: The interim arrangements for IPTRs should be phased out and replaced by the Peer Approved Clinical System as soon as possible.

Introduction of PACS

32. The transition from IPTR to PACS was due to take place in May 2014. RDUK understands that the Scottish Government have delayed the introduction of PACS in order to pilot the scheme in the first instance. It should be noted that formal guidance on PACS has yet to be issued publicly and that there are no defined timescales for its introduction. As a result, there is a degree of uncertainty amongst patient organisations about what PACs will look like in practice, how it will operate and how patients will be involved in the process.

33. Genetic Alliance UK acknowledge that it is in the best interest of patients for a pilot scheme to be tested and for a robust system to be introduced to ensure transition between systems is smooth. However, we would welcome greater communication from the Scottish Government regarding the progress of the current pilot scheme and the strategy for phasing out IPTRs and introducing PACS. Details of operational guidance for PACs should be made public as soon as possible and information on timescales be shared to allow patient organisations to prepare for this transition. Training on the new system must also be provided to both clinicians and patient organisations so that they can provide accurate information and support to patients.
Recommendation 13: The Scottish Government should communicate details of the PACS Pilot and the strategy for implementing PACs in Scotland, including timescales for implementation.

Recommendation 14: Training and guidance on how PACS works must be provided to clinicians and patient organisations ahead of its implementation to ensure they are equipped to support patients through the process.

Expert Clinical Opinion

34. During the Health and Sport Inquiry in 2013, the importance of the appropriate expert clinician being involved in the IPTR/PACS process, was cited. Genetic Alliance UK believe that decisions relating to the access to medicines should be based on the clinical opinion of experts in the specific condition. It must be noted that due to the nature of rare diseases, it may not be possible to locate a suitable expert clinician from within Scotland and as such IPTR panels must look outwith, to the rest of the UK and EU.

35. It is also considered that decisions made by clinicians who are not experts in the specific rare condition are likely to underestimate the seriousness and impact of the condition, and may also have unrealistic understandings of the course of deterioration of affected individuals.

36. A solution that may address the lack of specialist clinical expertise in rare conditions at health board level is to encourage IPTR/PACS panels to call on the expertise of a list of experts similar to that used by the SMC in gathering evidence for its decisions. This list of experts should include details of specialist centres for rare diseases, even where these exist outwith Scotland or the UK. Should this be implemented, serious consideration would need to be given to the criteria used to determine whether an individual is an appropriate expert, as well as to how best to both encourage experts to contribute their time to decisions made about patients they are not directly treating.

Recommendation 15: Whenever possible, IPTR/PACS panels must feature an appropriate clinical expert in the condition, whether that be by telephone or in person.

Recommendation 16: Health Boards should consider developing a comprehensive list of experts in rare diseases, similar to that used by SMC.

New Medicines Fund (NMF)

37. Genetic Alliance UK does not support the use of ring-fenced funding to maintain access to medicines and services for rare diseases, or for any group of patients. We believe that the best means to ensure that all patients are able to access effective medicines and services on the NHS is by establishing a system for evaluating and commissioning them that is timely, transparent and fair to all. Ring-fenced funds make it more difficult
to adapt resource allocation to patient need. Once a ring-fenced budget is put in place, it becomes much harder to justify either increasing or decreasing the amount of money allocated for that purpose. This means that is possible for either too little or too much of the limited NHS budget to be spent on one group of patients or type of condition. As a result, those patients whose medicines or services are financed by the fund may not receive the care or medication they require, or alternatively, they may receive disproportionately more while other patients, whose medicines or services are not paid for by the fund, lose out.

38. However, Genetic Alliance UK welcomes the Scottish Government’s commitment to ensuring funds are available to support access to medicines in Scotland. Whilst the New Medicines Fund has likely improved access to medicines for rare diseases in Scotland, the NMF lacks transparency on its use.

39. To date, no operational guidance on the NMF has been published and patient groups report having a limited understanding of exactly how the NMF works in practice.

40. In addition, there is a lack of clarity on how much of the NMF is being spent and what it is being spent on. We appreciate that for many rare diseases, figures on allocations of funds may not be able to be released due to policy restricting publishing information on medicines accessed by less than five people with a rare condition. However, in the interest of transparency, the Scottish Government should release regular reports detailing how the NMF is being operated, accessed and spent.

Recommendation 17: The Scottish Government should regularly produce a report on the breakdown of spend under the NMF.

41. It is understood by Genetic Alliance UK that in 2015/16, there have been changes to the way in which the NMF is allocated, with funding being allocated to Health Boards. It is the opinion of RDUK that the NMF remain a single ring-fenced fund rather than allocated to individual health boards. Being run centrally, ensures that the NMF can be monitored and cannot be absorbed by Health Board budgets or the underspend be used for matters not related to its intended purpose.

Recommendation 18: The NMF should be retained in its previous form as a single ring-fenced fund, rather than being allocated to local health boards

Conclusion

42. Genetic Alliance UK would like to commend both Scottish Government and SMC for their continued commitment to improve access to medicines for rare disease in Scotland. Since the Health and Sport Committee published its findings in 2013, transparency and public involvement in SMC have improved considerably. The steps taken to date appear to have contributed to an increase in approval rates, although it is difficult to assess whether this is a result of the changes that have been
implemented. Whilst acknowledging important changes at SMC and IPTRs, it is also important to recognise that there is still scope for greater change and to increase access to new medicines in Scotland.

Summary of Recommendations

SMC

- Recommendation 1: The role of public partner at SMC should be reviewed and consideration given to increasing the opportunities for patients to provide their perspective in person.

- Recommendation 2: SMC Public and Patient Involvement Team should hold regular training days.

- Recommendation 3: SMC should consider how to share best practice relating to patient involvement in the SMC process.

- Recommendation 4: Membership of the NDC and SMC should include suitably trained patient representatives.

- Recommendation 5: Patient representatives that have participated in the PACE process for a specific medicine should be invited to attend the SMC meeting to answer any questions raised by SMC members.

- Recommendation 6: SMC must ensure appropriate expert clinicians are involved in decision making

- Recommendation 7: Expert clinicians involved in PACE should be invited to attend the SMC meeting to provide clarification or answer questions.

- Recommendation 8: SMC should evaluate the significance of the QALY in SMC decisions for rare disease medicines.

- Recommendation 9: SMC should consider applying greater flexibility when assessing rare medicines and consider removing the QALY from decision making for rare medicines.

- Recommendation 10: Research to monitor the impact of PACE statement on decision making should be undertaken

IPTR/PACS

- Recommendation 11: The interim arrangements for IPTRs must be monitored to ensure exceptionality is not a factor in decision making.

- Recommendation 12: The interim arrangements for IPTRs should be phased out and replaced by the Peer Approved Clinical System as soon as possible.
• Recommendation 13: The Scottish Government should communicate details of the PACS Pilot and the strategy for implementing PACs in Scotland, including timescales for implementation

• Recommendation 14: Training and guidance on how PACS works must be provided to clinicians and patient organisations ahead of its implementation to ensure they are equipped to support patients through the process.

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**New Medicines Fund**

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