



## **Not if but how - Impressions from Market Access for Cell & Gene Therapies Congress, 18-19th October, London**

This new two-day conference allowed for honest discussions in small groups helped by the simple set-up and high-quality contributions from both payers and industry. Our head of market access, Nicolas Touchot chaired a keynote panel on Preferred Payment Models for cell and gene therapies. The news of Kite's CAR-T US approval coming in at \$373,000 additionally stimulated positive discussions on day two. There was goodwill and consensus on the payer/manufacture objective to get to a 'fair deal' and the need for a 'win-win' BUT the 'devil' is in working out what exactly this means. Here are our impressions from the two days highlighting the enormous promise but also the enormous challenges still to overcome.

### **Managing investor and manufacturer uncertainty**

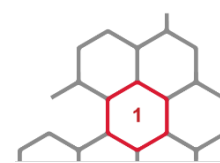
Only the weight of future approvals and the underlying data on efficacy and safety of novel gene therapies will lead payers to seriously consider and eventually commit to new payment models and managed access schemes at country level. A 'one size fits all' model appears elusive but the iterative nature of this process will over time lead to greater certainty on reimbursement. This is helped by >70 late stage developments in the pipeline looking to gain access to patients. Investors and manufacturers won't be holding their breath just yet however, there is no quick break-through anticipated. Assessing gene therapy treatments, often for unknown, rare diseases can be complex and time consuming. Manufacturers can help this process by offering comprehensive and well supported material with a clear rationale while, as much as possible, aligning all stakeholder data needs.

### **Risk pools or special funds?**

There were no suggestions from payers that, in the short term at least, more healthcare budget will be put aside to serve the reimbursement of the anticipated wave of novel gene and cell therapy treatments. However, in single payer or even multi-payer systems, risk pools remain a possibility. Similar, in principle, to an improved UK cancer drug fund (CDF) or in multi-payer-systems an improved German Morbi-RSA (abolished 2009), to ring fence access to treatments bearing greater uncertainty than traditional medicines.

### **Are payers ready?**

Highly cost-effective treatments can still bust current budgets and are therefore not necessarily affordable. Payers understand the challenges but are not close yet to proposing a universal method for reimbursement of treatments where the value is realised over time but remains uncertain. Currently, most payer budget cycle horizons are around two years and may stretch to three to five years at best. While the pace of coming up with new models seems sluggish, NICE put forward the possibility of 'fleeting foot adjustments' in some cases, whereby other cases e.g. orphan diseases with endpoints based on surrogate markers will take time to assess. In Germany, the 'tools' needed for managed access are not there yet



either, 99% of existing contractual arrangements are simple rebate contracts, some with a cap. To allow for managed access the whole system will have to change.

Payers also have to learn about the meaningfulness of novel endpoints in many of the rare diseases put forward for treatment. This was illustrated recently by the FDA asking for an advisory committee's opinion on the clinical meaningfulness of the MLMT endpoint seen with Luxturna (voretigene neparvovec) from Spark Therapeutics Inc. adding to the time taken for the approval and reimbursement process.

As this conference summary is being finalised, NICE has published its 31-page **consultation document** on Strimvelis, which reads relatively positive overall but also illustrates the complexity of analysis required prior to any gene therapy reimbursement decisions.

### **Managed Access Schemes**

While managed access schemes seem the preferred way of payers, who are overall reluctant to deal with uncertainty, they are not a panacea. Payers in reality much prefer simple one-time payments avoiding having to follow a patient over time and collecting complex data. However, it was argued that mandatory patient registries paid for by the manufacturer as part of managed access may be attractive in addressing both managing uncertainty and fairness towards the manufacturers. The technical difficulties in successfully tracking patients over time presents a similar challenge in both the US and Europe.

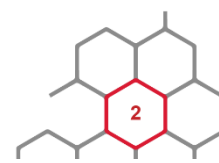
Manufacturers claim that a 'fair deal' must allow not only for prices to go down or even disinvestment but also to go up if real world evidence supports a better price. This was verbally acknowledged by payers. The industry and their investors hope to see this being implemented in writing too in the near future.

Managed Access Pathways follow the context of regulatory Adaptive Pathways. These are attractive but it was acknowledged that there is much to learn over the coming years. This learning may involve payers listening to issues such as manufacturing complexity and manufacturers taking note of 'wrap-around-costs' or structural costs when a novel therapy changes the existing care infrastructure and requires investment in areas that add to the mere drug therapy.

### **Are manufacturers ready?**

It will be the manufacturer's responsibility to explore sufficiently what payers will pay for in different countries, the evidence needed to show this and to design their trials and data set accordingly. Assuming annuity payments can be agreed, manufacturers are likely to receive a certain amount per successfully treated patient over time from payers. Risks the manufacturer has to manage include incurring a potential loss during the early years or potential deductions from an annuity if a patient requires additional therapy if this has been agreed as part of a risk-sharing agreement.

Other manufacturer paid investment into real world evidence may include building registry infrastructure and managing those through intermediaries. Additionally, costs could arise in



other areas, where payers feel that manufacturers should not only share a financial risk but also actively participate in setting up new treatment infrastructure and processes.

## Conclusions

With more future approvals for rare and more common diseases anticipated, and no additional funds being added to the drug-budget, reimbursement will be driven even more by the quality of the data supporting payer value and the difference that a proposed treatment can make to the Quality of Life of a patient.

As one of the manufacturer delegates put it “The complexity of market access has surpassed the complexity of regulatory approval”.

Manufacturers recommend meeting with HTA bodies early and taking advantage of early consultation frameworks such as **EUneHTA** in order to both harmonise evidence requirements (while prioritising the more detailed national advice over higher level EU advice) and realising cost efficiencies between multiple stakeholders.

Furthermore, while starting with a smaller but high-value-adding indication for proof-of-concept, payer reluctance to indication or value-based pricing puts more emphasis on manufacturers getting their launch sequence right. ‘Ultrarare’ diseases with fewer than 100 patients per country remain a viability challenge for payers and manufacturers – commercially very difficult. On the other hand, assuming there are 6,000 rare diseases with an estimated prevalence of 0.5-1/100,000, adding them all up makes for 5% of the population – too many to be ignored.

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