Dear colleagues,

Please find below minutes from a very interesting discussion which took place at the EUCOPE webinar.

Kind regards,

**EUCOPE Webinar: How can Europe remain at the forefront of innovation to the benefit of patients with rare diseases?**

**Date: 8 July 2020**

Participants of the webinar agreed (somewhat agree (37%)/completely agree (36%)), that given the experience of COVID in the EU it is vital that EU supports home-grown companies developing OMPS from original research.

**Question 1:** In general, it is recognized that the OMP has been a success to support the development of OMPS in the long term. However, more recently, since 2016, Europe has seen radical drops in the designation and development of OMPS. Further, EU-based small to mid-sized companies focused on developing OMPS are simply not profitable. What can and should the EU do with regards to this situation?

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**DG SANTE, Medicines: policy, authorisation and monitoring**
- The orphan and paediatric evaluation assesses how the system works since 2000.
- Many factors influence the orphan landscape and thorough analysis is needed before any conclusions can be made on the presented decline of designations in the period from 2016-2019.
- Since 2000, there was an increase in OMPS. Compared to US, there are higher numbers, however, there are differences in eligibility criteria to obtain orphan designation both in EU and US.
- The evaluation shows a decline of new therapeutic areas, only 1 out of 5 orphan designations is for new areas today.
- 64% of designations needed to demonstrate significant benefit, which shows that while the Orphan Regulation was there to promote medicines where there are no therapeutic options, now we see clustering around some therapeutic areas.
- However, we still see increase in authorised orphan medicines.
- Importantly, SMEs are in the heart of any policy and orphan incentives are available in the current system for SMEs. Particular attention to SMEs will be given in the policy options which we will consider.
- Pharma strategy will give a holistic view on how to address different issues the EU pharma policy is facing, including the acute problem of access.

- JW Scannell Analytics Ltd.;

- The last 15 years there was a shift towards orphan and oncology products.
- Medicines are paid by Americans. There is a big price difference for the same medicine in US and EU.
- Companies/investors are concerned about this difference in price.
- Launching medicines in Europe will decrease because of discussion for more transparency and from fear that the US will use the lower prices in Europe as benchmark for setting prices in the US.

- PTC Therapeutics (Company only developing orphans)
- Optimistic about the future.
- Some products from PTC Therapeutics pipeline will be first treatments in the area.
- Duchenne product is the only approved in the EU since 2014 (Translarna).
- According to the company the price is not the problem causing issues with access.
- R&D environment and incentives are what is important for the companies.

**Question 2:** One of the challenges of OMPs is that their economic impact is not quantified nor measured long-term, compared to their short-term costs. Many caregivers are actually parents forced to
leave the workforce, essentially costing society twice. In the case of CAR-Ts for ALL, we see that hospital costs are radically reduced when compared to traditional bone-marrow transplants, yet, these impacts are not evaluated or quantified as part of the HTA process. How can we better account for the impact of effective OMP treatments long-term?

**Mechanism of Coordinated Access to Orphan Medicinal Products (MocA)**
- EU could do a lot: support the use of Real World Evidence (RWE), post-authorisation evidence generation.
- Benchmarks for cost-effectiveness are needed. IT and infrastructure to check the claims made by applicant.
- Joint HTA makes sense for orphan medicines, as they are often the cutting edge (patients participation, new technologies used).

**World Duchenne Organisation**
- Current HTA processes are not designed for orphan medicines.
- Reimbursement models: the system is not ready to manage gene therapies with high costs.
- Data is an issue. We need to break the silos. Data sharing should be ensured even before the clinical steps.
- Digital outcomes are critical, especially in crisis like Covid-19.
- RWE generation is needed.
- Joint HTA and joint reimbursement needed. Now the market is segmented.
- In 2012 only in 4 countries the cost to the system was 2b euro. Assessing properly orphans would help health systems.

**DG SANTE, Medicines: policy, authorisation and monitoring**
- Access and affordability is one of the main pillars of the Pharma Strategy
- Evidence alignment from Regulators and HTA bodies needs to be discussed.
- The Commission HTA proposal aims to boost cooperation amongst EU Member States for assessing health technology and address these issues.
- Commission has already been doing work in recent years with support from the EU health programme and conducted by the OECD to help Member State exchange best practices on outcome-based payment schemes and managed entry agreements in general.

**PTC Therapeutics**
- One assessment is needed, which applies to all MS.
- HTA process does not take into account some societal benefits e.g. cost benefits for carers.
Global pharma investment decisions: US and China are bigger and preferred markets for industry. The European pharmaceutical market will grow more slowly in the coming years.

Comment from the audience: More collaboration between EMA and FDA needed

DG SANTE, Medicines: policy, authorisation and monitoring
- A lot of effort is ongoing in this regard already and a good collaboration is already established between EMA and FDA. Alignment of standards also at international level.

World Duchenne Organisation
- Duchenne, 1 product approved in EU and not in US, and one products approved in US and not EU. More harmonisation needed and joint scientific advices are a golden opportunity.
- Biomarkers approvals in US and clinical outcomes in EU does not help in closing the gap.

Mechanism of Coordinated Access to Orphan Medicinal Products (MocA)
- EU wide HTA assessment would help the companies when US product is placed on EU market and quickly accessible to all patients

PTC Therapeutics
- Standard approach with clinical trials is impossible in many cases, number of patients is too small. RWE and registries is the answer.

What one key deliverable would you like to see concretely committed in the next year to help maintain both EU competitiveness and patient access related to OMPs?

Define policy options for the orphans review and build on the pharma strategy, with the involvement of all stakeholders.

More innovation for the benefit of patients.

Orphan medicines pricing more opaque and less transparent. The more transparent the prices are the more difficult it will be in relation to US prices and will keep innovation away from Europe.

European cooperation on HTA needed.

Access is needed. R&D, fragmentation of funding, new models needed, academia and industry work closely, new models of assessing orphan medicines (basket trials).
Conclusions by EUCOPE, main messages from the webinar:

- Innovation new paradigm needed.
- Disease registries and innovative payment models needed.
- Biomarker indication should also be allowed in EU.
- No changes in legislation needed.
- Competition is needed for therapeutic areas where treatments already exist but we need to remember about the areas without treatment options.
- Industry should discuss how to achieve better access of medicines for EU patients.