EUCOPE

European Confederation of Pharmaceutical Entrepreneurs AISBL

8 July 2020, 11-12AM CET

EVENT REPORT

BACKGROUND

On 8 July EUCOPE hosted a webinar on the Future of Rare Disease Innovation in Europe elaborating on an Office of Health Economics analysis on the challenges of developing therapies for rare diseases which was recently unveiled at the 10th European Conference on Rare Diseases (ECRD).

The webinar brought together a multi-stakeholder group of experts for a lively debate and exchange of perspectives on rare disease and Orphan Medicinal Products (OMPs).

The objective was to discuss existing and new trends which impact the rare disease community and expected developments in the lead up to the publication of the OMP and Paediatric Regulations evaluation.

SPEAKERS

- Olga Solomon, DG SANTE; European Commission
- Anna Bucsics, Mechanism of Coordinated Access to Orphan Medicinal Products (MocA)
- Jack Scannell**,** JW Scannell Analytics Ltd.
- Thomas Bols, PTC Therapeutics
- Dimitrios Athanasiou, World Duchenne Organisation

Host: Alexander Natz, EUCOPE

Moderator: Duane Schulthess, Vital Transformation

Alexander Natz, Secretary General of EUCOPE kicked off the event stressing the importance of the moment we are going through, in view of the ongoing Pharmaceutical Strategy consultation process and the upcoming publication of the evaluation on the OMP and Paediatric Regulations. He noted how a larger part of society has come to recognise the pharmaceutical industry as one of the drivers for the economy and its importance to the economic recovery, in light of the pandemic.

Regulation on OMPs had an evident positive impact on research, development and innovation to the benefit of rare disease patients, but more needs and can be done.

The discussion among the panellist started with a survey question to the audience:

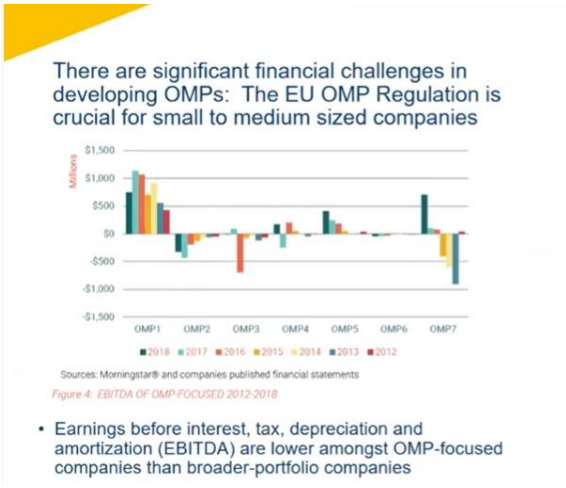
> '*On a scale of 1 to 5, with 5 being 'Completely Agree', how strongly do you agree with the following statement:*
>
> *"Given the experience of COVID-19, it is vital that Europe supports home-grown companies developing OMPs from EU original research.'"*

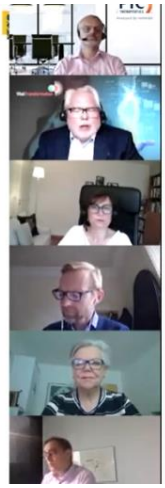
Results of the poll showed that around three quarters of those that responded somewhat or completely agree with this statement.

Vital Transformation showed a graph which illustrates the decline over the last 20 years in EU designation and authorisation in comparison with US trends. However, **Olga Solomon, Head of Medicines: Policy, Authorisation and Monitoring Unit at DG SANTE** stressed the importance of contextualising the data and trend within a multifactorial environment. A thorough analysis is needed before arriving at conclusions. The comparison with US would be an oversimplification because the evaluation criteria to obtain the marketing authorisation - and almost the entire system - are different. For instance, it can happen that some orphan products are available in the EU but not in the US and others in the US but not in the EU. This year there are already 10 new authorised orphan products in the EU.

The European Commission is now working to make a clear pathway for pharmaceutical companies by evaluating the factors that influence and contribute to the development and accessibility to OMPs.

Ms Solomon reiterated that small to medium-sized enterprises (SMEs) are at the heart of every policy the European Commission is taking and they are extremely important to the development in the field.





However, there is room for improvement. Patient access to new orphan medicines is highly varied across Europe, as remarked by **Thomas Bols, representative of medium-sized companies (PTC Therapeutics**); not all countries have access to medicines authorised in the EU. He also shed light on the incongruencies with the European Commission on the terminology used for defining SMEs. Overall, he expressed optimism towards the future of rare disease innovation in the EU. Yet, some OMPs with authorisation are not accessible however, unlike general beliefs would have us think, there is not a direct correlation with the wealth of a country and availability. The explanation instead is found in different reimbursement systems. He hopes to see more dialogue between European authorities and Member States.

The speaker representing the investment sector, **Jack Scannell (JW Scannell Analytics Ltd)** stressed the fact that the EU is lagging behind the US and the Asian markets because the approval pathway is not attractive, while in the US the sector deploys sizeable capital.

Anna Bucsics, Project Advisor to Mechanism of Coordinated Access to orphan medicinal products – MoCA, outlined how an integrated approach between Health Technology Assessment (HTA) and legal frameworks is very much needed to support EU competitiveness in the area of rare diseases to the benefit of patients. The entire system needs to be better integrated with multi-stakeholder interactions for achieving tangible outcomes.

Building on these arguments, the patient representative **Dimitrios Athanasiou (World Duchenne Organisation)** stressed the pivotal role of data, Real World Evidence (RWE), and patient reported outcomes (PROs). Evidence and interoperability of data contribute to breaking down siloed work and make integrated assessments possible, that ultimately shape reimbursement systems. The importance of having RWE and disease registries was an area of consensus among the panellists. Disease registries can support ground-breaking discoveries in R&D, better understand the outcomes and improve patient safety. On this aspects, Olga Solomon reminded that, ultimately, some aspects are Member States' competence and the right environment needs to be in place. She highlighted that there is currently a lot of work underway to encourage the use of RWE, but that there is a need to "learn from this and build a system that integrates RWE".

According to the last data available, since 2000, the number of authorised orphan medicines increased from 8 to 169 and has benefitted about 6.3 million patients in the EU. This was possible because the

Regulation has successfully incentivised companies to invest in the development of OMPs to address the unmet need of rare disease patients. Without such incentives, it would not be possible. Indeed, SMEs have the peculiarity of having a limited product portfolio and this makes their business volatile, with little or no return on investment.

Collectively, the 252 SMEs involved in orphan medicine development employ 8,739 people. Of these, 77% are employed in SMEs that were created after the OMP Regulation came into force in 2000. Indeed, over 25% are employed by companies created since 2014 with a clear positive impact on European economy.

Still, as speakers remarked, the EU can become more attractive to investors. Investment decisions however have to be considered together with ongoing global trends and overall industrial policies.

Building on the current cooperation between FDA and EMA is important to fill knowledge gaps and encourage a streamlined approach to products approval for rare diseases.

In most of the cases, there is no need to modify the current legislation. Much can be improved within the current regulatory framework.

To conclude the meeting each speaker shared **key points they would like to see moving forward to foster rare disease innovation to the benefit of patients.**

- **Thomas Bols, PTC Therapeutics**: all the strategies and policies the Commission is putting forward with the possibility for all stakeholders to comment on them to achieve a balanced approach between access, affordability and innovation.
- **Olga Solomon, DG SANTE**: More innovation to the benefit of patients and policy actions building on the evaluation of the OMP regulation
- **Jack Scannel**, JW Scannell Analytics Ltd: Make sure that Europe with its market requirements can compete with the more competitive conditions the US has to offer
- **Anna Bucsics, (MocA):** a major step forward in EU cooperation on HTA and cooperation on managed entry agreements
- **Dimitrios Athanasiou, World Duchenne Organisation**: tackle fragmentation of capital, more cooperation and support to research coming from academia and industry, new kind of clinical trials e.g. basket trial. We need innovation mostly in our thinking – a paradigm shift in mentality is key.

Alexander Natz summarised the outcomes of the meeting and gave some final remarks: Cooperation between payers and industry in the current framework is possible, working on disease registries and innovative payment models e.g. for CAR-t is already happening and more discussion at EU level can only further these cooperative efforts.

Video recording available here