EUCOPE

European Confederation of
Pharmaceutical Entrepreneurs AISBL

5 March 2019, Brussels

EU PARLIAMENT BREAKFAST EVENT ON GENE & CELL THERAPIES

EVENT REPORT

On 5 March 2019, EUCOPE, in cooperation with SME Connect and SME Europe, organised a breakfast policy debate **on Gene & Cell Therapies (G&CTs) entitled "*From discovery to access – how can we ensure Europe is at the forefront of innovation and access delivery of gene and cell therapies?"***. Held in the European Parliament in the presence of 50 stakeholders from the EU Parliament, the EU Commission, Permanent Representations of EU Member States, patient representatives and industry, the event aimed at **raising awareness on G&CTs as well as the associated challenges of delivery of access to these treatments**.

Opening the policy debate, **Dr Paul RUEBIG MEP** (EPP, Austria, President of SME Europe and of STOA) outlined the importance of the technological achievements and of developing new treatments and making important scientific discoveries across Europe, concluding on the essence of **strong collaboration between experts and the people in need of these new therapies**.

Alexander NATZ (EUCOPE, Secretary General) then drew attention to the uniqueness of G&CTs, in **improving the quality of life of people living with severe diseases,** including genetic cancers and rare diseases. After explaining the diversity of G&CTs and numerous areas of applications, not only to treat diseases but also to alter their course, EUCOPE's Secretary General alluded to the expected increased availability of G&CTs in Europe by 2025, with **more than 1000 G&CT clinical trials worldwide.** Acknowledging the European achievements in setting up a predictable legislative and regulatory

environment, with EU Regulation n° 1394/2007 on Advanced Therapy Medicinal Products and Directive 2011/24/EU on the application of patients' rights in cross-border healthcare, Mr Natz highlighted some of the specific challenges faced by companies in delivering treatments to patients across Europe, such as:

- At present, **only ten centres specialised in G&CTs exist in Europe**, implying that **patients often need to travel to other EU Member States in order to get the right treatment**. There remain major disparities in the application of the Cross-Border Healthcare Directive amongst Member States.
- **Several countries lack the necessary expertise** to deal with such treatments and technologies, which widens the gap among Member States.
- There are widely varying approaches to **payment models across the EU.**

In his conclusions, he called for the development of innovative and harmonised payment models (e.g. allowing payments in instalments) in Europe, which can contribute to broadening the access to such treatments.

In her intervention, **Irene NORSTEDT** (Acting Director for Directorate Health and Head of Unit Innovative & Personalised Medicine at DG Research & Innovation, EU Commission) **stressed the impact that research can have on patients' lives**. As an example, she mentioned the story of a young Syrian boy living in Germany who suffered from junctional epidermolysis bullosa (JEB) with 80% of his skin falling away and who could be cured through an innovative gene therapy developed by a group of Italian researchers. The research which reached this astonishing result was supported by EU funding. She also pointed out that the **EU dedicates significant funding to research, including G&CT area.** Among some of the instruments which allow to improve research cooperation at the EU level, she recalled the **European Reference Networks** (ERNs), the **International Rare Diseases Research Consortium** (IRDiRC) and the **European Joint Programme on Rare Diseases** (EJP RD). Irene Norstedt finally referred to the establishment of new modus operandi between DG SANTE and DG RTD towards increased synergies on the different programs active in various fields of R&D.

Maria KELLEHER (Director of Market Planning at BioMarin) praised the progress the European research hubs and networks, driving advancements in several fields, including G&CTs. She, however, stressed the importance to address remaining **challenges**, such as **the great differences at EU level in clinical trial infrastructure and approval pathways**. She finally hinted at the need for **more efforts to ensure that there is easier and broader access for patients to these new innovative treatments**. In fact, the **potential of G&CTs to be transformative should be recognised**, as they allow benefits to health systems, patients and the society as a whole.

Karolina HANSLIK (Senior Project Manager at EURORDIS Rare Diseases Europe) declared that **EURORDIS is closely following the development of innovative therapies for patients**, especially those deriving from G&CT research, that are currently in clinical trials or about to enter the market. After presenting the **RARE IMPACT initiative** led by EURORDIS, a patient-focused collaboration to assess the challenges along with proposing actionable solutions, Ms Hanslik added that **EURORDIS calls for the need to make these live-saving treatments affordable for everyone** (without patients relying on alternative ways to afford the needed cure, e.g. crowdfunding) **and always available when required**. In conclusion, she mentioned the importance of ERNs, calling them an excellent initiative which should allow more effective circulation of knowledge and expertise on rare diseases throughout Europe.

After the above-mentioned keynote speeches, **the need for more digitalisation in the EU national healthcare systems was outlined,** in order to facilitate cross-border data exchange. In this regard, **Ms Norstedt recalled the EC efforts to promote the use of the Electronic Health Records** by EU Member States. Regarding digitalisation, she added that innovation comes also from the development of mobile apps able to detect diseases, to track health parameters and much more, which should be put under a regulatory framework. The **European Commission is committed to soon establishing a minimum standard of digitalisation that EU Member States should meet**.

Another aspect which was drawn to the attention of the audience was **the need to generate and collect robust patient-level data,** as there is a lack of information and data for a great number of diseases (especially rare diseases). **Real World Data (RWD) is equally important**; despite the criticism often related to RWD, there is the need to educate on the added value of real data. **Pharmacovigilance is also essential, especially in the long term,** as safety should always be a top concern for all stakeholders.

As for hospital exemptions, it was highlighted by Mr Natz that, while these exemptions are important and useful in many cases, they **should not be put in place when there is no added value of this practice** and it aims only at allowing economic savings, subsequently posing serious challenges to the industry.