Finding cures for the incurable

Advanced therapies are no longer the stuff of science fiction. **Martin Banks** reports on the regenerative therapies often referred to as "living drugs" and the hurdles currently impeding their availability in Europe

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The increasing prevalence of chronic diseases in Europe represents a high social and economic burden. Direct healthcare costs soared by 50 percent during the last decade and reached €1.5tn in 2017 - a staggering 9.6 percent of Europe's GDP. This was one of the main messages to emerge from a debate in the European Parliament on advanced therapies. The event was organised by RESTORE, a project seeking to promote implementation of advanced therapies which is partly funded by the EU's Horizon 2020 programme. Hosting the event, German EPP member Christian Ehler highlighted the economic costs involved in achieving excellence in science, technology and innovation. While putting forward several "instruments" designed to reduce the administrative burden for researchers and SMEs, he pointed to current budgetary constraints at Member State level and also the need to provide affordable medicine.

Hans-Dieter Volk of the Berlin Institute of Health said that advanced therapies "hold the promise of not just treating disease symptoms but providing cures for previously incurable diseases." Such regenerative therapies are already a reality, he told the audience, but cautioned that "affordability and accessibility" remain "huge barriers" to their widespread and sustainable availability in Europe.

"We now need concerted action to make these advanced therapies accessible to all in need by addressing scientific, technical, logistical and economic roadblocks," Volk said. To do this, he suggested that a European Research Area on Advanced Therapies could be setup, as well as a dedicated EU-funding programme for advanced therapies that integrate innovative elements, disruptive research and breakthrough innovations. He acknowledged RESTORE's efforts to create a sustainable European "eco system" and predicted that advanced therapies had the potential to "have a major impact on patients and society." Another keynote speaker, Mir-Farzin Mashreghi, group leader of the "Therapeutic Gene Regulation" group at the German Rheumatism Research Centre, noted that advanced therapies require the knowledge of the "molecular identity of each single cell", either driving or regulating the pathogenesis of chronic diseases. "Such information is essential for the selection of cells which qualify as living drugs. Single-cell sequencing technologies are key for understanding the real cellular composition of chronic diseases and are game changers, not only for therapies aiming to cure chronic diseases but also for therapy response."

As current therapies rarely cure, but merely fight symptoms, lifelong treatment is required, the debate was told. But this can result in potential adverse effects, limited quality of life and increased costs. Higher health costs or restricting access to new therapies "to a few wealthy patients" are not solutions, it was said. Guillaume Fusai, who is responsible for EU affairs at INSERM, a French public scientific and technological institute, also paid

tribute to RESTORE, saying it is a "unique European hub to accelerate innovation." He said that by advancing knowledge about life and disease, treatment innovation and public health research, such expertise will boost the development of advanced therapies and living drugs. "By mixing industrials, hospitals and research organisations RESTORE will speed up innovation and new patient treatments." The debate also heard directly from a young patient who had suffered chronic kidney failure but, as a result of the advanced therapy treatment she had received and the kidney donation from her mother, is now well on the road to recovery. She said, "I take just one pill per day and am now able to lead a pretty much normal life."

Petra Reinke, a professor of internal medicine, nephrology and transplantation, said, "Currently, we need lifelong therapy and, in the case of transplant patients, 20 to 30 pills per day, 365 days per year, for years and years. Most of these are for treating side effects." She added, "Reshaping the immune balance by the single application of regulatory T cells gives patients a new life as demonstrated here with our patient: one of the first transplant patients worldwide to receive the therapy. These are the cases and people who motivate us as physicians." Ivana Cattaneo, public affairs director for the European Region at Novartis Oncology, said that the pharmaceutical industry "recognises the challenges and opportunities of advanced therapies, whose complexity requires new models of collaboration." She added, "All along the whole trajectory from discovery to access, there is a need for all stakeholders to engage and collaborate in a different way."

Also contributing to the debate was Zami Aberman, executive chairman of the Israeli company, Pluristem, who predicted that emerging technologies will enable advanced therapies to become cost-effective drugs which would help millions of patients in need. "These technologies have the potential to significantly impact Europe's ecosystem by providing cost-effective therapies to many unmet medical conditions and generate advanced research centres and biopharma companies that will use advanced tools such as AI and machine learning to improve patients' health while reducing costs for healthcare systems." The debate also heard from Karolina Hanslik, of the health NGO EURODIS, who said that in Europe an estimated one in 2,000 people suffer from a rare disease - about five percent of the population. She said the majority of these people living with a rare disease has "delayed or no access at all" to the medicine they need. "Often the necessary medicine simply does not exist." She concluded with the message that the rare diseases community "expects to be supported by the EU institutions, national governments, manufacturers and clinicians," adding, "Our ambition is to increase by three to five times the approval of new rare disease therapies." ★

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