EMA-EuropaBio Bilateral meeting

Video conference
5 May 2021
## EuropaBio Representatives

<table>
<thead>
<tr>
<th>Name</th>
<th>Title/Position</th>
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<tr>
<td>Claire Skentelbery</td>
<td>Director General of EuropaBio</td>
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<td>Andrew Toppen</td>
<td>Chair of the EuropaBio Board, Head of Public Affairs Europe &amp; Global Business Franchises at Novartis</td>
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<td>Neil Mulcock</td>
<td>Chair of the EuropaBio Healthcare Biotechnology Council; Vice President Government Affairs and Policy EMEA at Gilead</td>
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<td>Aoife Gallagher</td>
<td>Vice-Chair of the EuropaBio Healthcare Biotechnology Council; Head European Government Affairs at Eli Lilly</td>
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<td>Christiane Abouzeid</td>
<td>Chair of the EuropaBio Regulatory Policy WG, Head of Regulatory Affairs at BioIndustry Association (BIA)</td>
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<td>Pedro Franco</td>
<td>Vice-Chair of the EuropaBio Regulatory Policy WG, Director Europe for Global Regulatory &amp; Scientific Policy at Merck Serono</td>
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<td>Laura Liebers</td>
<td>Lead on clinical trials within EuropaBio Regulatory Policy WG, Director, International Regulatory Policy &amp; Intelligence at Vertex Pharmaceuticals</td>
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<td>Bernard Grimm</td>
<td>Healthcare Biotechnology Director at EuropaBio</td>
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<td>Violeta Georgieva</td>
<td>Legal Affairs Manager at EuropaBio</td>
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Introduction to EuropaBio
EuropaBio Mission

To be the recognised voice of the European biotech community championing innovative solutions for society’s challenges
Innovative biotechnology for people and planet

European citizen health...
• World class ecosystem
• Delivering for patients
• Innovation continuum

...in a sustainable world
• Across sectors
• Industrial process
• Health environment

Technologies
Regulation
Policy advocacy
Communication & celebration

Innovation pathway
Market access
SME support

SME Platform

Policy advocacy

Industrial Biotech Policy Priorities
Focus on biotechnology with...
EuropaBio Healthcare Council

Health innovation in our DNA
EuropaBio healthcare members spend € 60 Billion a year on R&D 20.8 % of revenue

A new treatment of cancer or a rare disease is 72% likely to come from an emerging biopharma company

Over 50% of our members are active in advanced therapies

EuropaBio also represents 19 National Biotechnology Associations and Life Science Clusters, whose combined membership amounts to over 2500 companies mainly SMEs

Over 500 innovative medicines developed by EuropaBio’s members with over 650 in development
EuropaBio Healthcare Council
How are we organised

Healthcare Council

ATMP Working Group
Regulatory Working Group
Orphan Medicines Working Group
Digital/ Data Working Group
Biosimilar/ Procurement Working Group

Biopharmaceutical Strategy Group

Patient BioForum
European leadership in life sciences and biotechnology at stake

What is at stake: who will lead on the next wave of strategically important technologies?

Past technologies: electricity, car, antibiotics, social media, artificial intelligence, 3D printing, gene therapy, hydrogen fuel cells, liquid biopsies, neurotechnologies.

Future technologies: semi-conductors, digital platforms, autonomous vehicles, CRISPR-cas, quantum computing, new batteries, graphene.


EU lead (& Asia, EU): GPS, smart phone, blockchain, Internet of things.

Who leads?:

Examples taken from WIPO, MIT, WEF, OECD, etc.
Despite Covid, the funding gap remains

Funding gap for scaling up highly innovative startups and SMEs

US venture capital investments are 4-5 times higher than EU

Source: [Invest Europe, Pitchbook]
How do we create more EU biotechnology unicorns?

What is at stake: will Europe be home to future «unicorns»?

Number and market value of “unicorn” companies (valued at over €1 billion) by regions

Source: CB Insights (Jan 2021)
What’s holding back European innovation?

**Innovation performance**
- Strong research performance not translated into innovation
- Lack of breakthrough/disruptive innovations that create new markets

**Innovation funding**
- Financing gaps (2 “valleys of death”) in
  - Transition from lab to enterprise
  - Scaling up for high-risk innovative start-ups

**Innovation ecosystem**
- Many national & local ecosystems, but fragmented at European level
- Need to include all regions and all talent (especially female)
Horizon scanning
Biotechnology pipeline
Key trends
The Bio Revolution has started

Domain and examples

Human health and performance

- Health optimization in future generations
- Gene drives to reduce vector-borne diseases
- Cell-, gene-, and RNA-based approaches to prevent, diagnose, and treat diseases
- Improvements in drug development and delivery

Arenas of innovation

- Biomolecules
- Biosystems
- Biomachine interfaces

Transformational capabilities

- Increased control and precision
- Enhanced ability to engineer and reprogram human and non-human organisms
- Increased throughput and productivity of R&D
- Growing potential for interfaces between biological systems and computers

Key trends on the biotechnology pipeline

The pipeline is highly innovative, with half being new substances and cell and gene therapies gradually gaining importance.

Indication expansions vs new products:
- 52% New product
- 48% Indication expansion

Orphan vs non-orphan drugs:
- 59% Orphan drugs
- 41% Non-orphan drugs

Key categories:
- Biological: 37%
- Radiation therapy: 4%
- Small molecule: 26%
- Biomarker identification: 2%
- Cell therapy: 56%
- Vaccine: 1%
- Diagnostic: 1%
- Gene therapy: 1%

Almost 50% of therapies in development are new products, among which lower incidence, previously omitted diseases are gaining interest (and investment), with 40% of the pipeline being orphan drugs. More than 90% of products in the pipeline are biologics and small molecules. However, the share of Next-Generation Biotherapeutics (NGB), such as cell, gene, and nucleotide therapies in clinical development continues to increase. In years 2014-2019 the number of NGB products has more than tripled, as they have high potential especially in previously intractable diseases.

Source: IQVIA EFPIA pipeline report 2021
Cell and Gene therapies

MORE THAN

100

DISEASES BEING EXPLORED FOR
POTENTIAL TREATMENT WITH CELL
AND GENE THERAPIES

362

CELL AND GENE THERAPIES
IN DEVELOPMENT

132

A THIRD OF CELL AND GENE
THERAPIES ARE IN DEVELOPMENT
FOR RARE DISEASES

MEDICINES IN DEVELOPMENT | 2020 UPDATE

CELL AND GENE THERAPY

Nearly 400 Cell and Gene Therapies in Development Target a Broad Range of Diseases

Source: American BioPharmaceutical Medecines in Development report
Pipeline by disease and phase

Medicines in Development by Disease and Phase

Source: American BioPharmaceutical Medecines in Development report
In Gene therapies alone

Figure 1. Gene therapy pipeline volume, preclinical through pre-registration phase, 1995–2018

Note: Annual volume snapshots are captured in May of each year.

Source: Pharmaprojects®, August 2018
Increase in clinical trials

The volume of initiated clinical trials has increased year on year since 2015 with oncology having the most extensive pipeline

Full pipeline – number of trials started in 2015-November 2020

Pipeline summary – key therapeutic areas\(^2\) [# trials started in 2020]

- Oncology
- Infectious Disease
- Neurology
- Hematology\(^*$\)
- Respiratory
- Dermatology
- Endocrinology
- Cardiovascular
- Psychiatry
- Ophthalmology
- Gastrointestinal
- Rheumatology
- Women’s Health / Sexual Health
- Allergy / Immunology
- Hepatology
- Nephrology
- Other (*) incl. Onco-haematology

The volume of pipeline activity has continued to increase over the last years. Assuming that in 2020 the average number of clinical trials launched per month will remain as in the period of Jan 2020 – Nov 2020, total number of clinical trials started in 2020 will be similar to 2019 (-3%).

Source: Clarivate Analytics Cortellis, Aug 2020 (TA’s share) and Nov 2020 (total number of trials); Phase II includes Phases II, IIa, IIb, Phase III includes Phase IIIa and IIIb. Terminated trials were excluded from the analysis. Trials not industry sponsored and device trials were excluded; (1) Total number of trials started in 2020 to be defined – final number will be available in the beginning of 2021; (2) Data from August 2020
With ~40% of the oncology pipeline in Phase 1, this therapy area is expected to witness significant changes over the next 3-5 years.
Clinical trials geographic split: Europe losing ground?

- Checkpoint inhibitors combinations
- Gene therapies
- Nash
- MRNA vaccines
- Novel HIV therapies
- HBV

Source: IQVIA EFPIA pipeline report 2021
Increasing share of Asia in clinical trials

The impact of COVID-19 on trials geographic split is not visible to date; increasing share of Asia in clinical development is observed

Full pipeline – trials started in 2010-2020 per region:
- Northern America
- Asia
- Europe
- Africa and Middle East
- Oceania

European pipeline – trials started in 2010-2020 per sub-region:
- Eastern Europe
- Southern Europe
- Northern Europe
- Western Europe
- Share of clinical trials run in the UK in the total number of trials in Europe

The geographic distribution of clinical trial location has not changed significantly compared to previous year, which indicates that the COVID-19 pandemic has not forced pharmaceutical companies to move their development activities. The long-term trend observed is the increasing share of clinical trials conducted in Asia (mainly China, South Korea), which grew from 14% in the years 2011-2013 to 24% in 2020.

Source: Clarivate Analytics Cortellis, Nov 2020; Phase II includes Phases II, IIa, IIb. Phase III includes Phase III and III. Terminated trials were excluded from the analysis. Trials were industry sponsored and device trials were excluded; (1) including Caribbean; (2) including Georgia; (1) Please note that II trial may be run in several locations — resulting in differences in total number of trials versus number of trials in geographic split.
In summary, several innovation areas have appeared on the horizon, with a potential to gain importance in the coming years.
...whereas other interesting technologies are further on the horizon, worth keeping an eye on in the coming years.

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<th>PROTACs</th>
<th>Gene editing technology</th>
<th>Exosome therapy</th>
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<td>PROTAC (proteolysis-targeting chimera) substances have been developed as a useful technology to degrade and dispose of targeted proteins that support cancers. New therapies could target different cancer types, potentially offering higher efficiency than inhibitors, at the same time causing less undesired toxicities and side effects.¹</td>
<td>CRISPR/Cas9 is a gene editing technology that is applicable across disease areas – cancer, infection, muscular dystrophy, etc. Works by injecting a DNA construct, which targets a defective gene, replacing it with a functional gene⁴. CRISPR-Cas9 system has a potential to be faster, cheaper, more accurate and efficient than other genome editing methods³.</td>
<td>Exosomes are nano-vesicles released by nearly every cell in the body, which may be used as a diagnostic/therapeutic agents. Pre-clinical research has shown that exosomes reduced scar tissues caused by a heart attack⁴. Exosomes may be a next step in stem cell therapies, offering an improved safety profile⁸.</td>
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**Senolytics**

New class of drugs that are able to induce death of senescent cells responsible for aging and age-related diseases⁶. Targeting aging itself might be a novel strategy to prevent a number of conditions that elderly patients struggle with. First-in-human trial of senolytic drugs are preliminary, but encouraging.

**Microbiome therapy**

Microbiome therapy aims to restore healthy gut microbiota to control a variety of local and distant pathologies⁷. This field continues to garner interest from the pharmaceutical industry with extensive research, funding and multiple collaborations. With new advances in machine learning and diagnostic techniques, microbiome research will likely keep growing and becoming more data science-driven and precise.

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¹ ScienceDirect; ² Nature.com; ³ Genetics Home Reference; ⁴ NCRI; ⁵ Exopharm; ⁶ NCRI; ⁷ EFPIA; ⁸ Abbreviations: link to glossary

Innovations included in original shortlist – more details available in the附件}

Source: IQVIA EFPIA pipeline report 2021
Creating the future Innovation Access Model

- Patient central role
- Early access to regulatory advice
- RWE to address clinical uncertainties
- Reward innovation through new value assessments
- New care delivery models
- New payment models