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Opinion of the European Economic and Social Committee

The European Life Science Strategy along with the Strategy on Research and Technology infrastructures with focus on the treatment of rare diseases

(exploratory opinion requested by the Cyprus Presidency of the Council of the EU)

(C/2026/2541)

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1. Conclusions and recommendations

The European Economic and Social Committee (EESC):

1.1. welcomes the Strategy for European Life Sciences, and the special focus that Cyprus places on rare diseases; stresses that ensuring a holistic approach and nourishing environment can make Europe a global leader in the life science (especially for rare diseases and genomics) and backs the call of the European Parliament for an EU Action Plan for Rare Diseases to be swiftly adopted, including by reviving and further developing the Tartu call for a healthy lifestyle and embracing the 'One Health' approach;

1.2. calls for the swift alignment and clarification of how 'rare diseases' are defined, in order to support the coordinated implementation of EU initiatives in this area (including the European Strategy on Research and Technology Infrastructures and Choose Europe). Research and technology infrastructure (including that focused on rare diseases) is a key enabler for the development of the life sciences and the longevity economy. It should cover not only medicine and genetics, biotech, care infrastructure and pharmacy, but the whole longevity culture (including prevention, healthy food, lifestyle, balneo treatment and recreation, physical and mental health, and many other elements) and the full 'life sciences' spectrum, stretching beyond the diagnosis and treatment of diseases (including rare diseases). Cross-cutting support actions should include the promotion of talented and young entrepreneurs, data integration, IP protection and innovation financing in the 2028-2034 multiannual financial framework (MFF);

1.3. stresses the key role of the EU in coordinating investment in research, infrastructure (especially for diagnostics) and skills while respecting the competences and specificities of the Member States, so as to endeavour to excel in the life sciences and reduce heterogeneities and inequalities in accessing good medical services; with this in mind, suggests that the holistic approach involve: comprehensive scientific and data-based methods to diagnose and understand the causes of diseases (especially rare diseases, e.g. genetics, unhealthy habits and lifestyles, pollution, stress, etc.); disseminating information on prevention and early diagnosis (e.g. pre-conception); screening and effective and accessible treatment; and high-quality care and accessible support infrastructure for patients and their families. These areas should be boosted through the targeted development of research and innovation (R&I), accompanied by sufficient funding, a simpler, future-proof legislative framework anchored in reality and the promotion of competitiveness, excellence, talent and digitalisation;

1.4. underlines that European partnerships are vital in fostering the meaningful and structured engagement of the full spectrum of stakeholders and asks for formalised mechanisms for their participation. Structured, transparent and systematic involvement of patient organisations, social partners and civil society organisations, including their mandatory representation in governance bodies, will enable structured consultation in defining priorities and data protocols, transparency requirements on decisions and data use, and a mechanism ensuring that their contributions are effectively integrated.

1.5. recommends that translational centres, specialised in rare diseases, be built in each Member State so as to gather the medical community, patients, social workers and biobanks, and ensure that they are coordinated and supported at EU level. These structures can be used to build a more structured approach in tackling rare diseases and to set clearer goals and priorities, applying the holistic and integrated approach in practice. Strengthening the European Reference Networks (ERNs) and increasing their outreach is a vital part of this process.

1.6. underlines that the EU life sciences are evolving into data-driven sciences. Therefore, the development of the European Health Data Space (EHDS), 1+MG genomic and BBMRI-ERIC biobanking infrastructure and the ELIXIR bioinformatics ecosystem is critical to delivering precision medicine, genomic diagnostics, molecular epidemiology and translational research.

2. **General comments**

2.1. As health systems in the EU differ considerably (in clinical practice, care pathways, data maturity, population needs and governance structures), the EU should adopt a model to prioritise coordination rather than uniformity, using regional specificities to test, validate and scale health innovations.

2.1.1. The EU must support the alignment of interfaces, coordination of objectives and structured exchange of evidence and good practices, ensuring interoperable data flows and comparable clinical and regulatory evidence. To achieve this, key enablers such as EHDS-compliant data environments, interoperable evidence standards for digital health, AI tools, Advanced Therapy Medicinal Products (ATMPs) and diagnostics, and shared methodological frameworks for Joint Clinical Assessments/Joint Scientific Assessments (JCAs/JSAs) are needed. ATMPs, including gene, cell and tissue therapies, are among the most significant therapeutic groups for rare diseases, particularly those of monogenic origin. Their development should be treated as a strategic EU priority. ATMP test beds and clinical sandboxes could form a network of specialised nodes that supports experimentation in diverse healthcare contexts and enables validated innovations to scale across the regions.

2.1.2. The European Medical Agency (EMA), health technology assessment bodies and 'notified bodies' should coordinate evidence requirements, early dialogue and consultations more quickly and improve the flexibility of standardised procedures and regulatory sandboxes. This will ensure that innovations can be evaluated under local conditions; that evidence travels, even if practices differ; and that regional and clinical JSAs/JCAs feed into EU-level understanding.

2.2. Genomics and molecular biology constitute the scientific foundation of contemporary life sciences in the EU and are increasingly embedded in clinical practice, public-health surveillance and R&I partnerships. Whole-genome and -exome sequencing and transcriptomic approaches (RNA sequencing) enable both precision medicine and the identification of genetic disorders. Genomics (studies and clinical diagnostics) should be integrated as a cross-cutting component of EU health and research cooperation. National coordination points should monitor sequencing infrastructure, research capacity, workforce skills and data integration readiness, ensuring alignment with national health strategies, digitalisation efforts and the 1+MG initiative.

2.3. The EESC supports a holistic approach to the life sciences and the One Health framework, recognising 'the interconnectedness of human, animal and environmental health'. Early diagnostics, screening, the promotion of R&I, healthy lifestyles, prevention and improved access to care are vital prerequisites for longevity, equity and improving pathways for patients. The MFF 2028-2034 should reflect health priorities, including rare diseases, to avoid dilution once they are integrated into the ECF and Horizon EU.

2.4. The ERNs constitute a key component of the EU system for managing rare diseases, enabling the consolidation of expert knowledge, cross-border clinical consultations and data sharing. Their reinforcement and integration with the EHDS and genomic initiatives should be considered a strategic priority, so as to offer better and easier access, and to foster collaboration between national healthcare systems. They should act as hubs for cross-border genomics-enabled care by promoting the integration of clinical-grade sequencing workflows, secure pipelines for variant interpretation and multidisciplinary genomic boards, and mechanisms for the structured sharing of de-identified case data, including genomic and phenotypic information, across ERN centres, in compliance with data protection rules and EHDS.

2.5. The EESC reiterates its suggestion to extend the Health Emergency Preparedness and Response Authority (HERA) mandate to cover rare diseases, or to use HERA as a model for a new EU authority for non-communicable diseases. The aim is to foster coordination, boost solidarity in tackling rare diseases and help develop and speed up the adoption and implementation of an EU action plan on rare diseases. It also calls for Orphanet to be strengthened so that it can publish its work in all EU official languages and ensure that patients and professionals have the information they need.

3. **Specific comments**

3.1. *Rare diseases*

3.1.1. Rare diseases affect approximately 36-39 million people in EU, with an estimation of 6 000-8 000 distinct disorders identified. Around 70-80 % of rare diseases are genetic in origin. Due to small and dispersed patient populations and high diagnostic complexity, coordinated actions at EU level are required to improve diagnosis, research, care pathways and access.

3.1.2. Rare disease patients often experience a 'diagnostic odyssey' lasting on average between five and seven years in Europe, frequently preceded by misdiagnoses. Genomic diagnostics significantly reduce this delay, improving access to appropriate therapy and support.

3.1.3. Access to rare disease therapies varies across EU. Differences in reimbursement, HTA procedures and health system organisation generate territorial inequalities. Harmonised HTA frameworks, joint clinical assessments and strengthened cross-border mechanisms are essential for ensuring equitable access.

3.1.4. The EESC calls for commitment to interoperable data formats, harmonised consent models and metadata standards for rare disease data, in line with the EHDS. Common standards for genomic data generation, biobank sample processing, metadata models and mechanisms for linking records between biobanks and clinical registries are vital in order to harmonise biobank procedures and integrate genomic data into biobanking and registry infrastructure relevant to rare diseases. Cross-border DACs should work on unified evaluation criteria, ensuring predictable and fair access for academic, clinical and industry researchers.

3.1.5. The EESC stresses that only a holistic, integrated approach and a culture of cooperation and collaboration can bring about success in developing the life sciences. This involves also better integrating natural and climate-based therapies into prevention and rehabilitation. The EU's network of medical spas, climate health resorts and natural therapeutic environments offers evidence-based balneology and climatotherapy that complement biomedical innovation, support mental health and rehabilitation, and help reduce long-term healthcare costs. The European Commission must recognise these assets as part of the Strategy for European Life Sciences, promote research and digital tools relating to them, and link them to broader objectives on healthy ageing, prevention and the green transition.

3.1.6. Innovation potential is increasingly constrained by structural regulatory barriers that delay market access, increase compliance costs and reduce the EU's attractiveness as a location for research and investment. Core obstacles that hinder innovation must be removed, including by:

— streamlining and simplifying overly lengthy, complex and duplicated authorisation processes (often taking two to three years), addressing the lack of proportionate and risk-based pathways;

— removing inefficiencies in the pre-submission, risk-assessment and risk-management phases;

— scaling the disproportionate requirements under the Transparency Regulation;

— providing robust impact assessments and proportionate rules and adequately considering the effects of new legislation and administrative procedures on competitiveness and SMEs; and

— incentivising Member States to apply mutual recognition in order to ensure a shared minimum level of compatibility across Member States.

4. **Towards Europe's global leadership in the life sciences**

4.1. To address the challenges related to the diagnosis and treatment of rare diseases, the EESC suggests focusing on:

— establishing individual patients pathways;

— early and effective diagnosis, including genetic screening and preconception testing, in cooperation with biobanks and rare disease organisations;

— the translation of scientific advances into therapeutic solutions by accelerating approval pathways, including for orphan drugs;

— the training of specialists (clinical genetics specialists and counsellors);

— improving public understanding of rare diseases, early detection and prevention and the overall culture of taking the pursuit a healthier lifestyle to a new level;

— ensuring sustainable, long-term funding and the coordinated development of genomic research and infrastructure, including sequencing centres, bioinformatics platforms, secure cloud environments and reference databases;

— aligning paediatric and adult treatment pathways to ensure a lifecycle approach.

4.2. The EESC stresses the need to modernise the legislative framework and facilitate market and global access for biotech products, taking into account the broad spectrum of health-related biotechnology, including products, technologies and industrial applications. Regulatory requirements should be designed to reflect recent scientific developments and to enable and facilitate innovation.

4.2.1. Continued support to, advanced and high technology such as vaccines, CAR-T cell therapies and personalised medicine as major drivers of global competitiveness is needed. It is equally important to maintain a strong biotechnological foundation (especially regarding immunology and oncology), and to acknowledge the vital role of biosimilars ([1]) as established therapeutic options for chronic and life-threatening diseases, which contribute significantly to patient access and healthcare system sustainability. The EMA confirms the clinical equivalence of biosimilars to reference products. Clearer, predictable and efficient rules tailored to patient needs and the realities of biotech innovation can boost the advancement, development and application of health-related biotechnology (including ATMPs, molecular diagnostics and novel therapeutic modalities) with a view to excelling in prevention, diagnostics and treatment. The widespread use of biosimilar medicinal products in high-expenditure therapeutic areas can free fiscal space and increase the capacity for reimbursing innovative therapies, including ATMPs.

4.2.2. Enabling innovation requires cutting red tape and simplifying, especially when it comes to accelerated approvals that can benefit from clearer, more predictable and streamlined pathways. To that end, the work of the EMA and the EU network of regulatory authorities should be fostered, including to enhance and speed up regulatory recognition where non-EU authorities are involved. To help accelerate innovation in R&D (especially pharmaceuticals), ensure easier implementation of alternative (non-animal) testing methods (e.g. cell-based assays) and encourage replacement of in vivo tests with ethical, scalable alternatives, the validation and acceptance process for animal-free models should be simplified. Further, the Member States should be incentivised to update pricing and reimbursement frameworks to reflect the specific nature of biotechnology development and production (upfront investment, long development cycles and substantial risk), striking a balance between cost-efficiency, market access and supply security pathways.

4.2.3. Further efforts to convince the Member States to address systemic inefficiencies in clinical trial authorisation and management ([2]) are needed in order to ensure faster approvals for clinical trial applications and less administrative burden when submitting amendments to ongoing trials (substantial modifications, including parallel submissions). Mutual trust in the assessment of reporting by Member States and coordination among ethics committees throughout the EU should be fostered so as to improve the harmonisation of the approval process for multinational clinical trials. Regulatory efficiency must go hand in hand with scientific integrity and competitiveness, ensuring continued patient safety while enabling fair market entry for high-quality medicines (including biosimilars).

4.2.4. Standardising regulatory requirements is key in reducing uncertainty for developers and manufacturers of medicinal products. Considering the continued developments in biotechnology, the European Pharmacopoeia should include a broader set of biological and biotechnological substances, reflecting the diversity of modern biotech-based medicinal products in the EU. Regulatory gaps in GMP, e.g. in areas such as postbiotics, probiotics or cleaning validation, should be closed through targeted EU-level guidance, along with scientifically sound criteria that can help assess the comparability of substances.

([1]) Which play role in access to and stability of health systems, but are not breakthrough tech innovations.

([2]) Over the past five years, approximately 60 000 patients in the EU have missed participation in clinical trials due to administrative bottlenecks and fragmented approval systems.

4.2.5. The EU must leverage the interoperability of digital and AI technologies (via AI factories) to advance research and clinical development while ensuring secure data sharing and access. If implemented effectively, the EHDS and the AI Act could provide the private sector with access to critical datasets that unlock transformative innovation, provided intellectual property and trade secrets are safeguarded.

4.2.6. The expansion of the EMA's DARWIN-EU federated data network would enable broader use of real-world data in decision-making, on the condition that EMA and national authorities are resourced with top expertise and cloud-based infrastructure to optimise workflows.

4.2.7. Strengthening intellectual property protection and regulatory data protection, and ensuring efficient authorisations and audit processes (in research and manufacturing, e.g. import licences for biotech platforms, Substances of Human Origin and environmental permits) can support scale-up. Access to venture capital and instruments such as Important Project of Common European Interest, EIB financing, public procurement mechanisms and tax incentives can help bridge the gap between laboratory research and GMP-scale production.

4.3. Biosimilars must be recognised not only as cost-saving tools but as innovative products developed through highly complex biotechnological processes. The EESC stresses the need to ensure:

— long-term supply security and industrial resilience in the EU by moving public procurement practices concerning biosimilars beyond a purely price-driven logic (as focusing solely on the lowest price risks undermining the economic viability of biosimilar manufacturers);

— transparency, reduced development costs and enhanced reproducibility by further developing existing biosimilar guidance by therapeutic class, including models for PD comparability and the application of physio-chemical characterisation tools and reconsidering restrictions based on historical knowledge gaps to enable the broader use of efficient, evidence-based methodologies; and

— the establishment of a competence centre for pharmaceutical manufacturing, which can help attract and promote talent and is important for supporting innovation in industrial-scale production.

4.4. The Transparency Regulation ([3]) has introduced requirements that greatly hinder innovation, particularly for SMEs. It has substantially reduced the scope of the information that can be kept confidential, limiting applicants' ability to protect proprietary data. The process for requesting confidentiality is overly bureaucratic, adding major delays during validation even before scientific assessment begins. The excessive study notification obligation poses an additional challenge, involving risk of penalties or delay and hindering operations and compliance. The early disclosure of applications, whereby dossiers become publicly available at the start of the assessment, with only confidential sections withheld, presents another concern, as confidential sections are often limited to personal information and other narrowly defined elements. This allows competitors to identify ongoing innovations and gather intelligence to quickly catch up with the original applicant, enabling non-EU operators to launch competing products even before the EU applicant has obtained their authorisation.

4.5. The Health Claims Regulation ([4]) constitutes a major barrier to innovation. Because of the very low success rate, almost no new applications are submitted. The disproportionate and untenable scientific evidentiary thresholds established under the Regulation inhibit scientific advancement, discourage investment in research on the health benefits of foods, given the low chances of success, and limit consumer access to credible information. A revision of this framework is warranted to close the innovation gap between the EU and other parts of the world.

([3]) Regulation (EU) 2019/1381 of the European Parliament and of the Council of 20 June 2019 on the transparency and sustainability of the EU risk assessment in the food chain and amending Regulations (EC) No 178/2002, (EC) No 1829/2003, (EC) No 1831/2003, (EC) No 2065/2003, (EC) No 1935/2004, (EC) No 1331/2008, (EC) No 1107/2009, (EU) 2015/2283 and Directive 2001/18/EC (OJ L 231, 6.9.2019, p. 1, ELI: http://data.europa.eu/eli/reg/2019/1381/oj).

([4]) Regulation (EC) No 1924/2006 of the European Parliament and of the Council of 20 December 2006 on nutrition and health claims made on foods (OJ L 404, 30.12.2006, p. 9, ELI: http://data.europa.eu/eli/reg/2006/1924/oj).

Europe's network of medical spas, climate health resorts and natural therapeutic environments offers proven models of balneology and climatotherapy that accelerate rehabilitation, strengthen resilience and help reduce long-term healthcare costs. The EESC therefore calls on the Commission to recognise these assets as part of the Strategy for European Life Sciences, to promote research and digital tools and enhance their accessibility, to link them to broader objectives on healthy ageing and prevention and to actively integrate natural and climate-based therapies into structured prevention programmes and efficient recovery pathways.

Brussels, 18 February 2026.

The President
of the European Economic and Social Committee
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