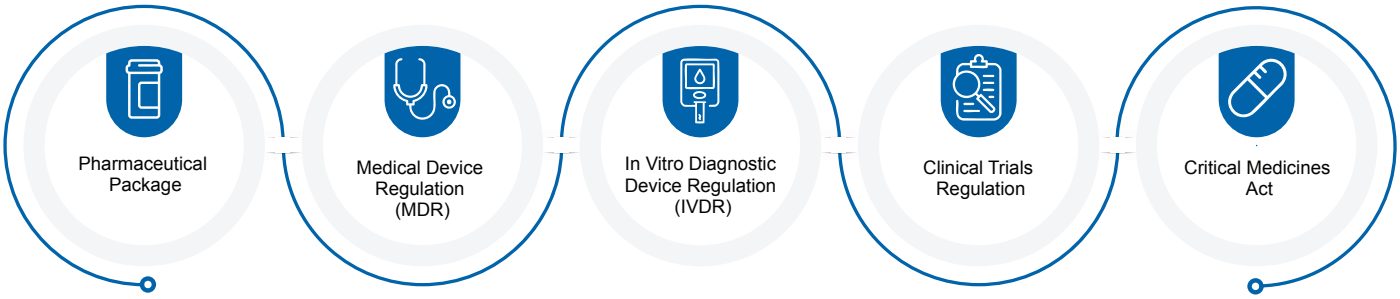
AMGEN

Amgen's suggestions for

COMMISSION PROPOSAL FOR A BIOTECH ACT

Biotechnology drugs provide important treatment options for patients with cancer, blood disorders, inflammatory diseases, cardiovascular disease, and many other illnesses. As highly complex molecules, they require specialised development, manufacturing, storage, and handling conditions[1]. They are usually administered as sterile products, requiring strict processes during production. The pharmaceutical regulatory framework (currently under revision) already allows biological products to be developed, manufactured and marketed as medicinal products. However, **there is a need for an overarching piece of complementary legislation that ensures that upcoming and ongoing initiatives work efficiently together in the context of biotechnology,** including:



It is essential that the Biotech Act **does not hinder or duplicate efforts from other pieces of legislation that already affect healthcare biotechnology, and at the same time allows for the flexibility needed to address the fast-moving knowledge and technical landscape** in which it operates, including upcoming digitalisation and the implementation of Artificial Intelligence (AI) and machine learning.

For Europe to stay competitive globally in attracting and promoting investment and innovation in biotech, Amgen has developed a series of recommendations to complement the existing and updated regulatory frameworks by reducing unnecessary bureaucratic processes, reporting and allowing faster access to innovative biotechnology medicines across the EU.

Regulatory asks

For innovative therapies to reach more patients as quickly as possible, we need a regulatory framework that is fit for this purpose.

1. Clinical trials in Europe: make Europe an attractive place to conduct clinical research for biotech products

1.1 Clinical trials: a centralised coordinated procedure for clinical trial assessment

The transition from the Clinical Trial Directive to the Clinical Trial Regulation has seen a move towards a coordinated assessment of clinical trials in the EU. While this progress is welcomed, gaps remain, particularly at the Member State level. The Biotech Act is an opportunity to build on the progress to date and future-proof the EU clinical trial system for anticipated innovations in the years to come. We believe knowledge and expertise from the clinical trial review process can be leveraged to facilitate marketing authorisation application (MAA) reviews, a process which, in the current EU set-up, is fragmented. We acknowledge there are several initiatives aiming to address these challenges, such as the ACT-EU pilots offering an opportunity for developers to receive scientific advice on clinical trials and the requirements for MAAs. To build on this progress, the Biotech Act should:

- Establish a centralised coordinated procedure for biotech products, under the supervision of the European Medicines Agency (EMA), building on the progresses to date. This will address the fragmentation in the current system and support continuity of knowledge building from trials through to marketing authorisation processes and even into the post-marketing setting.

1.2 Clinical trials: combination products requiring medicinal treatments and In Vitro Diagnostics (IVDs)/Medical Devices (MDs)

Innovative therapies that require the use of IVDs or MDs ("combination products") face significant hurdles, with regulatory roles and responsibilities sitting across a wide range of agencies and Notified Bodies under the current EU framework. Despite progress and centralization of processes through the Clinical Trials Regulation, there are still inconsistencies in the assessment processes across Member States, making multi-country clinical trials unnecessarily complex and delaying access to cutting-edge treatments for patients. To address these challenges, the Biotech Act should:

Establish a coordinated framework for combination products

- Introduce a unified process for the submission and assessment of clinical trials involving combination products.
- Include clear timelines and procedural steps without overlap and delays, ensuring alignment between requirements for medicinal products and their companion diagnostics or devices.
- Designate a single coordinating body or platform to oversee the joint review by national competent authorities for medicines and IVDs as applicable.

Standardise ethics review processes for combination products

- Include drug device combinations into an EU-wide framework/guidance for Ethics Committees' assessments to ensure uniform expectations and consistent interpretation of legislation across all Member States, helping with the delivery of multi-country trials.

2. Accelerated pathways

Biological therapies and innovative biotech products, including those targeting orphan diseases, often address areas of high unmet need. However, current regulatory timelines can delay patient access to these critical treatments. We therefore call for:

- An expedited timeline (e.g. 120 days), on top of the proposals in the Pharmaceutical Package to reduce the standard review timeline from 210 days to 180 days, for biologics with compelling preclinical and early clinical evidence of addressing unmet needs.

3. Resourcing

To meet the unique challenges of regulating biotech products, which often involve cutting-edge science and complex production methods, it is essential that regulators can keep pace with rapidly evolving technical knowledge. Therefore, an enhanced regulatory capacity with a biotech-specific focus will be needed for the future. This will ensure the EU remains a competitive leader in global biotech innovation while maintaining robust safety and efficacy standards. To accomplish this, the Biotech Act should:

Support with the specialisation of EMA experts

- Encourage Heads of Medicines Agencies (HMA) to build and coordinate a centrally managed biotech-specific pool of trained regulators accessible to all Member States, with expertise in areas critical to biotech innovation, such as advanced biologics; biomanufacturing, covering biologics production and scaling processes; and regulatory science for drug-device combination products. As a best practice example, we advise looking into the Paul-Ehrlich-Institute in Germany.
- Add Biotech matters to the Regulatory Training Academy under the coordination of the EMA supported by the HMA. As a best practice example, we advise looking into the IMPACT training process for CMC reviewers.
- Introduce a biotech scientific advisory panel within the EMA to provide consistent, expert guidance, and to be involved in the scientific advice process.

Provide funding for a system that continually works in future endeavours

- Create a Regulatory Innovation Fund to finance initiatives aimed at regulatory modernisation, such as regulatory sandboxes and pilot projects, with industry representation secured during the development of work programmes.
- Allocate appropriate funding to enhance and maintain databases, portals, and digital tools used in regulatory procedures.

Establish a regulatory modernisation initiative

- Establish a regulatory modernisation initiative for biotech products, appropriately funded by users-fees, to execute against biotech regulatory science programmes, with measurable outcomes.

Supply chain management

1. Repeated release testing after importation

Products imported into the EU from third countries must undergo full release testing (also called "import testing") before they can be distributed or sold. This testing must be conducted by an EU/EEA laboratory linked to a Manufacturing Importation Authorisation and listed in the Marketing Authorisation. However, testing can be waived under a Mutual Recognition Agreement (MRA) with trusted regulatory partners.

These requirements were initially introduced in the 1970s when global regulatory frameworks were less robust, and later formalised in the EU's pharmaceutical legislation, which is currently under revision. Import testing uses additional power and chemicals and generates waste impacting environmental sustainability. It also causes delays—especially for long-duration tests like sterility testing—reducing shelf life and limiting supply chain flexibility.

For trusted third countries with strong regulatory systems, these safeguards are now redundant. Modern quality controls, including Good Manufacturing Practices (GMP), Good Distribution Practices (GDP), Qualified Person (QP) certification, and regulatory inspections, ensure high standards across the manufacturing and supply chain. In light of this, the Biotech Act should:

- Allow reliance on release testing and the Certificate of Analysis (CoA) from the final manufacturing step in a country with a mature regulatory system. This should apply to imports from third countries recognised for strong regulatory oversight, based on inspection reports from trusted non-EU authorities.

2. Manufacturing processes

The current regulatory framework for the manufacturing of biologics and advanced biotechnology medicines manages risk and establishes rigorous oversight to ensure safety, efficacy, and quality. To foster innovation while maintaining these high standards, it is essential to reduce unnecessary regulatory burdens for specific modalities and refer to principles in clear, (and if needed) modernised guidance for manufacturers addressing which hazards need to be managed without deep-diving into technical details. To achieve this, the Biotech Act should:

Reconsider data requirements to capture the specificities of biotechnology

- Simplify and harmonize submission data requirements for manufacturing processes (highly critical for biologics where production involves living systems and complex processes) based on ICH M4Q(R2), recognizing the fact that data generated under processes described in the Quality Management System are well governed by regulatory inspections. Other aspects like for stock reporting are not different from small molecules and do not need further clarification.

Encourage the EMA to keep manufacturing guidance for biologics and biotechnology-derived products up to date

- Encourage the EMA to keep updating guidance on manufacturing processes if additional hazards must be adressed.

Protection of intellectual property rights

Biotech innovation requires significant upfront investment in R&D, often with extended timelines before commercialisation. Robust intellectual property (IP) strategies are essential to securing the financial backing necessary to sustain these long cycles. Small and medium-sized enterprises (SMEs) and startups, which drive much of the sector's innovation, often lack the expertise to develop and execute these strategies effectively. Moreover, the highly technical and transnational nature of biotech intellectual property protection presents unique challenges for effective enforcement across the EU's fragmented landscape. To address these challenges, the Biotech Act should:

1. Support to SMEs navigating commercialisation

- Establish specialised support services tailored to small and medium-sized enterprises start-ups in the biotechnology sector to strengthen their capacity to protect and leverage intellectual property (IP).

2. Strengthening collaboration on intellectual property protection

- Strengthen collaboration between the European Patent Office (EPO), and national IP offices to address cross-border biotech patent enforcement.

Reference

[1] For example, they are highly sensitive to temperature and must be stored and transported under controlled, cold-chain conditions to maintain their quality.

Amgen is one of the world's leading biotechnology companies, present in more than 100 countries and with a global team of 27,000 employees.

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