EHA position on the European Biotech Act proposal (2025/0406/COD)

September 25, 2026

The European Hematology Association (EHA) is the leading representative of hematology and hematology professionals in Europe. We are a not-for-profit organization with over 12,000 members. EHA develops and harmonises hematology education, produces clinical practice guidelines, supports career development and research, and advocates for affordable and equitable access to innovative therapies for patients with hematological diseases. EHA also founded the Coalition for Reducing Bureaucracy in Clinical Trials, a cross-disciplinary appeal by medical societies and patient associations in Europe.

European Biotech Act

Academic innovators (developers) play an indispensable role in delivering innovative, targeted treatments for European patients, but require dedicated, structural support. As it stands, acquiring funding for clinical research in advanced therapies is extremely difficult.

Strategic projects (Chapter II)

Due to the eligibility criteria for health biotechnology strategic projects (Article 3), academic institutions are unlikely to qualify. While strengthening the EU's competitiveness is important and warrants urgent action, **the Biotech Act proposal does not adequately support or incentivise academic and public-benefit research, nor promote equitable patient access.**

EU funding has predominantly targeted high-risk, high-reward biotechnology, which is often subsequently licensed to companies. As a result, some Member States effectively pay twice for the same advanced therapies—first through public research funding and later through reimbursement from constrained public health budgets—while many others lack access altogether. **EHA therefore calls on the EU to increase financial and structural support for non-commercial initiatives, including pivotal clinical trials, with the potential to benefit all Member States.**

Regulatory assistance (Article 34)

Academic developers also "often lack the regulatory expertise and capacity needed to identify, anticipate and plan their entry into the appropriate regulatory procedural pathways" and thus require appropriate support. Academic innovation often addresses (unmet) needs where there is no commercial interest. The support under Article 34 should therefore not be tied to 'strategic projects'. **The EU Health Biotechnology Support Network should also help academic developers with identifying and using the appropriate regulatory procedural pathway and regulatory support mechanisms.**

EHA welcomes the establishment of a Regulatory Status Repository (foreseen by Article 35).

Public investment with public return

As EHA stressed in its response to the call for evidence, public investment in biotechnology must be accompanied by tangible public benefit. EHA advocates for EU funding mechanisms that guarantee transparency and contribute to affordability and reinvestment in public health. This includes conditionalities that ensure publicly-funded innovation is accessible to patients and contributes to sustainable and timely healthcare delivery across the European Union. **Funding under the Biotech Act must be equipped with strong transparency, monitoring and reporting mechanisms.**

Impact assessment

The 'success' of the Biotech Act should be measured by its contribution to equitable and affordable patient access, as well as better health outcomes. EHA encourages the Commission to embed access and health impact assessments into the legislative framework. A stellar regulatory framework is of no use if the innovative treatments that are developed, assessed and authorised do not reach the patients who need them. The assessment should look at the strategic projects as well as the proposed SPC extension, as both will entail major public expenditure.

Amendments to Regulation (EU) No 536/2014 (Clinical Trials Regulation)

The Clinical Trials Regulation (CTR) requires targeted improvements to increase harmonisation, efficiency, and patient-centricity within the EU clinical research environment. The administrative burden faced by investigators is a major concern, impacting academic (public benefit) as well as commercial clinical trials.

Getting risk categorisation right is crucial to ensure that low-risk non-commercial trials—filling crucial gaps in research and improving patient care—are subject to proportionate requirements.

As Article 58 of the Biotech Act bundles all amendments to the CTR, references below follow the numbering in the Clinical Trials Regulation, as amended. All listed recitals are from the Biotech Act.

Key shortcomings in the CTR that still need to be addressed:

Risk categorisation and proportionate regulatory requirements

The Clinical Trials Regulation does not appropriately differentiate levels of risk within clinical research, subjecting low-risk studies to requirements designed for high-risk trials. The result is a disproportionate administrative and documentation burden, particularly for non-commercial sponsors, who conduct the majority of such trials (as stated in the proposed Biotech Act, Recital 138).

Non-commercial clinical research plays a critical role in advancing the scientific community's understanding of diseases, treatment approaches, and patient needs and experiences, thereby improving patient care and clinical outcomes.

Technical amendments on the next page.

Minimal-intervention clinical trials

EHA welcomes the new 'minimal-intervention clinical trial' (MICT) category, in Article 2(2), point (3a). However, MICT comprises a small number of treatment optimisation trials—namely, pragmatic comparisons of two authorised treatments, or trials addressing organisational or monitoring questions. **While the MICT designation is useful, it covers very few trials and does not address the regulatory burden for vital academic clinical research, including de-escalation trials.**

Low-intervention clinical trials

The importance of de-escalation trials

De-escalation trials address questions with major relevance to patients and health systems: reducing toxicity, treatment duration, hospital visits and costs, etc. These trials present opportunities to address the experienced physical, financial and time burdens. While crucial for patients, health budgets, and the evolution of science—this type of research has little commercial incentive and is therefore predominantly conducted by academia. Excessive regulatory requirements will render these (academic) trials unfeasible. Requiring established evidence for de-escalation studies is circular as their purpose is precisely to confirm whether e.g., a shorter duration or lower dosage retains sufficient efficacy. De-escalation trials can encompass one or a combination of elements: lower dose, reduced frequency, shorter duration, treatment discontinuation and response-adapted de-escalation.

The scope of low-intervention clinical trials

Article 2(2) must provide more clarity on the scope of 'low-intervention clinical trial' (LICT). **LICT should cover the aforementioned de-escalation studies, *as well as* comparisons of treatment approaches (mono or combination therapies) that are recommended by clinical guidelines or predominant in clinical practice—with an established safety profile.** A few examples: a randomization between different prophylactic antibiotics, the use of which is very well-established in clinical practice, or a trial comparing the use of vinblastine versus vincristine in the CHOP regimen, or a clinical trial comparing the DA-EPOCH and CHOEP regimens (both recommended by clinical practice guidelines).

Proportionate requirements

To ensure that LICT are subject to risk-proportionate requirements, these trials should be covered by the exception in Article 6(2a). An ethical review for de-escalation trials as well as trials investigating approaches from established clinical practice and/or recommended by clinical guidelines is risk-proportionate, and reduces the burden on sponsors, investigators and national competent authorities. The latter can then focus their limited resources on trials presenting a higher degree of risk and uncertainty.

Harmonised and future-proof

While provisions should be clear, EHA cautions against overly descriptive legal text, as it risks unintentional omissions and may not be future-proof. **EHA strongly recommends setting out the specificities of LICT in the implementation guidance, to be developed by the CTAG, so that the legislation remains fit-for-purpose and adaptable.** LICT should not exclude tumour-agnostic trial designs and umbrella trials.

EU guidance to ensure harmonisation and legal certainty

The Clinical Trials Coordination and Advisory Group should develop guidance to ensure that sponsors (and national competent authorities) understand which trials fall under the MICT and LICT categories. Practical examples should be included to support a harmonised interpretation and application of these designations, thereby reducing the risk of similar trials being classified differently across Member States. Clinical experts should be extensively consulted in this process. **Where necessary, the CTAG should develop guidance on the implementation of the adapted regulatory requirements for MICT and LICT, to safeguard harmonisation.**

Practical implications of the MICT and LICT designations

EHA supports risk-proportionate regulatory requirements for MICT and LICT:

- Adapted regulatory requirements throughout the lifecycle of MICT and LICT, in particular, with regard to the application dossier, authorization procedures, safety reporting and oversight – Article 3
- Adapted data submission requirements for part I of the application dossier – Article 25
- A simplified and risk-based approach to reporting requirements – Article 41
- The extent and the nature of the sponsor's monitoring should take into account the MICT or LICT designation – Article 48
- Simplified informed consent in (MICT) cluster trials – Article 30

However, in order to enhance legal certainty, more clarity is needed on the practical effects of these designations. For instance, wording such as *"the requirements (...) may be adapted"* in Article 25, and *"takes into consideration"* in Article 48 is too vague and will lead to disparate interpretations. **Where necessary, the CTAG should develop guidance on the implementation of the adapted regulatory requirements for MICT and LICT, to safeguard harmonisation.**

(Biotech Act, Article 58)
Regulation No 536/2014

- **Article 2, paragraph 2, point (3)**

European Commission proposal	EHA amendment
(3)'Low-intervention clinical trial' means a clinical trial which fulfils all of the following conditions: (a)the investigational medicinal products, excluding placebos, are authorised; (b)according to the protocol of the clinical trial, the use of the investigational medicinal product is evidence-based and supported by published scientific evidence on the safety and efficacy of those investigational medicinal products concerned; and	(3)'Low-intervention clinical trial' means a clinical trial which fulfils all of the following conditions: (a)the investigational medicinal products, excluding placebos, are authorised; ***(b) according to the protocol, the clinical trial investigates one or a combination of the following de-escalation elements:*** ***(i) lower dose,*** ***(ii) reduced frequency,*** ***(iii) shorter treatment duration,*** ***(iv) treatment discontinuation,*** ***(v) response-adapted de-escalation,*** ***(vi) other elements of de-escalation defined by CTAG guidelines;*** ***Or,***

(c)the additional diagnostic or monitoring procedures do not pose more than minimal additional risk or burden to the safety of the subjects compared to normal clinical practice in any Member State concerned;'	***the clinical trial investigates a comparison between treatment strategies that are established in clinical practice or recommended in clinical guidelines for the disease area concerned;*** ***(ba)the investigational treatment strategy is supported by relevant scientific evidence and/or clinical guidelines and/or established clinical practice that justify the study and characterise its foreseeable risks, including the potential risk of reduced efficacy;*** (c)the additional diagnostic or monitoring procedures do not pose more than minimal additional risk or burden to the safety of the subjects compared to normal clinical practice in any Member State concerned;'

- **Article 6, paragraph 2a**

European Commission proposal	EHA amendment
Notwithstanding paragraph 2, where the clinical trial is a minimal-intervention clinical trial, the assessment of the reporting Member State shall be limited to an ethical review by its ethics committee of the aspects referred to points (a) and (d) of paragraph 1.	Notwithstanding paragraph 2, where the clinical trial is a minimal-intervention clinical trial ***or a low-intervention clinical trial***, the assessment of the reporting Member State shall be limited to an ethical review by its ethics committee of the aspects referred to points (a) and (d) of paragraph 1.

- **Article 85, paragraph 5, point (j) – new**

European Commission proposal	EHA amendment
	To issue guidance on the scope of the the minimal-intervention clinical trial and low-intervention clinical trial designations, relying on input from clinical experts and patient representatives, to ensure an effective, risk-proportionate and harmonised implementation.

Harmonised and patient-centred informed consent

Whereas EHA supports the broader definition of informed (e-)consent as proposed in articles 2 and 29, **we call for stronger harmonisation requirements around informed consent**.

Harmonizing and streamlining ICFs with a two-part structure

While documentation in Part I of the assessment report constitutes a single submission, Part II requires separate submissions of different versions of the same documents from each Member State concerned (MSc).

This is because national ethics committees assess Part II according to their own national regulations, cultural contexts, and ethical standards—including the requirement for the informed consent form (ICF) to be provided in the local language. As a result, what is deemed acceptable in one country may not be in another, requiring customized ICFs for each country.

The lack of harmonization in ICF requirements not only undermines efforts to streamline the clinical trial application process, but also to create clear, patient-friendly ICFs, which are crucial for ensuring that patients fully understand the potential risks and benefits of participating in a trial.

The solution

- **The essential elements of informed consent, standardised across EU countries, should be included in part I of the assessment report, led by the RMS, and covered in Article 6.** This would include descriptions of screening tests, potential side effects, and anticipated benefits of the trial.
- **Country-specific details would be covered in part II of the assessment report, covered in Article 7.** Such details include available treatment options—which may vary based on the standard of care in each Member State—and legal information regarding protection of personal data and storage of biological samples.

Regulation No 536/2014

- **Article 6, paragraph 1, subparagraph a, point (iii) - new**

Clinical Trials Regulation	EHA amendment
(a)compliance with Chapter V as with respect to the following:	(a)compliance with Chapter V as with respect to the following: (...) ***(iii) essential and common elements of the informed consent form—applicable across the Member States concerned, and in accordance with the guidelines developed by the European Commission and the European Medicines Agency—including a patient-friendly description of the nature, objectives, anticipated benefits, risks and inconveniences of the clinical trial.***

- **Article 7, paragraph 1, subparagraph (a)**

Clinical Trials Regulation	EHA amendment
(a)compliance with the requirements for informed consent set out in Chapter V;	(a)compliance with the ***country-specific*** requirements for informed consent set out in Chapter V;

What is needed to make ICFs patient-centred

Article 29 CTR states that the clinical trial information provided to participants should be "comprehensive, concise, clear, relevant, and understandable to a layperson". However, the requirement to inform participants of all aspects relevant to their decision-making (Article 2, point 21) often leads sponsors to adopt an overly cautious approach, resulting in excessively detailed ICFs.

This creates barriers to participation for certain populations, leading to an unequal distribution of the risks and benefits of clinical research. The rules governing informed consent have become a substantial obstacle for many potential research participants, and should be revised to deliver shorter, simpler, harmonized and patient-centred ICFs.

EHA recommends:

- Using appendices for supplementary information (sections that are not crucial for patient decision-making).
- Shorter, more focused lists of the most common side-effects, avoiding technical language and using patient-friendly terms, and
- Using visual aids, including images, infographics and videos.

The aforementioned recommendations on informed consent are supported by the Coalition for Reducing Bureaucracy in Clinical Trials.

Regulation No 536/2014

- **Article 29, paragraph 2, point b**

Clinical Trials Regulation	EHA amendment
(b) be kept comprehensive, concise, clear, relevant, and understandable to a layperson;	(b) be kept comprehensive, concise, clear, relevant, and understandable to a layperson***, and, where applicable*** ***(i) include visual aids and infographics to facilitate understanding by the subject or their legally designated representative*** ***(ii) feature supplementary information that is not crucial for patient decision-making in the appendice(s)*** ***(iii) include a concise list of the most common side effects, avoiding technical language and using patient-friendly terms***

- **Article 29, paragraph 9 – new**

Clinical Trials Regulation	EHA amendment
	The Commission, in cooperation the European Medicines Agency, and after consultation with relevant stakeholders including medical societies and patient organisations, shall develop guidelines on informed consent to ensure a harmonised, patient-centred implementation of this Regulation.

Addressing challenges regarding the early termination of clinical trials

Ensuring due patient follow-up

The CTR does not provide adequate safeguards for patients in the event of early termination of the clinical trial. Under Article 37, sponsors are only required to notify Member States and to describe follow-up measures "when appropriate", which does not create a clear or enforceable obligation. This is particularly problematic when patients have already been treated. **EHA therefore calls for an amendment requiring sponsors to deliver adequate follow-up and posttrial care for all treated participants.**

Furthermore, Annex I (Part I, Section D) should require that clinical trial protocols include contingency plans for early termination. These should specify the responsibilities for follow-up, as well as the nature and duration of the monitoring. Such amendments are necessary to ensure that patient protection and fairness for healthcare providers are upheld.

Regulation No 536/2014

- **Article 37, paragraph 7**

Clinical Trials Regulation	EHA amendment
7. (...) In the case of early termination of the clinical trial for reasons not affecting the benefit-risk balance, the sponsor shall notify each Member State concerned through the EU portal of the reasons for such action and, when appropriate, follow-up measures for the subjects.	7. (...) In the case of early termination of the clinical trial for reasons not affecting the benefit-risk balance, the sponsor shall notify each Member State concerned through the EU portal of the reasons for such action and~~, when appropriate,~~ follow-up measures for the subjects. ***Sponsors shall devise a contingency plan for early termination, specifying foreseen follow-up and monitoring measures, to be included in the protocol.***

Regulation No 536/2014

- **Annex I, Section D. Protocol, point 17**

Clinical Trials Regulation	EHA amendment

	(ar) a description of envisaged measures for follow-up and monitoring of subjects in the event of early termination of the clinical trial.

Publication of trial outcomes

In addition, Article 37 should explicitly state that the obligation to submit a summary of results to the EU database also applies to trials that are terminated early. The failure to publish results from (discontinued) clinical trials is harmful both to patients and to medical science. It undermines the contribution made by patients, at personal risk, and prevents clinicians and researchers from learning from the outcomes. Over time, this leads to systematic gaps and biases in the evidence base, because studies with unfavorable or inconclusive results are less likely to be reported.

Regulation No 536/2014

- **Article 37, paragraph 4**

Clinical Trials Regulation	EHA amendment
4. Irrespective of the outcome of a clinical trial, within one year from the end of a clinical trial in all Member States concerned, the sponsor shall submit to the EU database a summary of the results of the clinical trial. The content of that summary is set out in Annex IV.	4. Irrespective of the outcome of a clinical trial, ***including where a clinical trial is terminated early,*** within one year from the end of a clinical trial in all Member States concerned, the sponsor shall submit to the EU database a summary of the results of the clinical trial. The content of that summary is set out in Annex IV.

EU platform for investigator communication with sponsors/CROs

Investigators currently face fragmented data entry and reporting processes across multiple EDC systems (or paper), which causes inefficiencies, duplicate work, and a considerable administrative burden.

EHA therefore proposes the development of a single EU-wide digital platform integrating EDC and safety reporting functions. Such a platform would enable the direct entry of participant data and adverse events, including streamlined reporting of serious adverse events (SAEs). It would also serve as a central communication hub, providing investigators with safety updates (e.g. SUSARs, ASRs) from sponsors and CROs in a single, accessible location.

In addition, the Clinical Trials Information System (CTIS) requires further functionality improvements to fully meet the requirements set out in Article 82 of the CTR. *These recommendations are supported by the Coalition for Reducing Bureaucracy in Clinical Trials.*

Reporting of serious breaches (Article 52)

EHA recommends amending this provision in order to create clarity and predictability for investigators regarding who determines what constitutes a serious breach.

The lack of clarity leads to discrepancies between clinical and regulatory perspectives and inconsistency in how breaches are identified, documented, and reported, potentially complicating regulatory oversight and diminishing accountability. A serious breach report should not be submitted without the investigator's agreement. To avoid conflicts of interest, three investigators should be consulted, at least two of whom would have to agree on categorising the deviation as a serious breach. *This recommendation is supported by the Coalition for Reducing Bureaucracy in Clinical Trials.*

Common EU clinical trial agreement template

Contract writing and negotiation are critical yet very administrative and time-consuming steps in the clinical trial process, that would benefit from EU harmonization. The lack of alignment, even at the national level, significantly contributes to delays in clinical trial initiation—especially in multinational trials and in certain Member States.

EHA strongly encourages CTR amendments to introduce a common EU clinical trial agreement template, supported by modular annexes for national adaptations. More harmonized contract writing will significantly streamline site-level negotiations. *This recommendation is supported by the Coalition for Reducing Bureaucracy in Clinical Trials.*

Role of the Reporting Member State (RMS)

EHA welcomes the strengthening of the Reporting Member State's 'leading role' (Article 4) and the clarification of its responsibilities throughout the text. These changes will be crucial for more predictability and harmonisation. We welcome the increased binding effect of the RMS Part I conclusion by restricting grounds for disagreement, as well as the exclusive right to request additional information from the sponsor between the validation and reporting date.

However, EHA supports **extending the RMS-led assessment model to include common, non-country specific parts of Part II of the assessment report.** In addition, we recommend the creation of training programs for the responsible teams within the Member States. *This recommendation is supported by the Coalition for Reducing Bureaucracy in Clinical Trials.*

Equitable patient access

While EHA recognises the importance of strengthening the EU's competitiveness, efforts to streamline and accelerate clinical trial processes must ultimately serve patients (Recital 123). The focus should therefore be on shortening "time to access", rather than "time to market" alone (Recital 122).

Impact assessment (Recital 159 and Article 97 CTR)
EHA welcomes the introduction of a review and monitoring process to assess whether the CTR amendments effectively support scientific and technological progress. However, the associated performance indicators should also evaluate whether these changes translate into a meaningful improvement in equitable access to innovative medicines for patients across the European Union. Patients must be recognised as the ultimate beneficiaries of clinical research, and regulatory changes should demonstrably deliver positive outcomes for them.

Improvements introduced by the Biotech Act that EHA welcomes:

Core dossier (new Article 27a)

EHA strongly supports the newly introduced 'core dossier' for clinical trials pertaining to the same investigational medicinal product. This constitutes an important step towards increasing efficiency and reducing the administrative burden. The depositary Member State, responsible for completeness and managing updates, is an important addition.

Fast-track for health emergencies (new article 14b CTR)

EHA welcomes the newly introduced "Accelerated procedure for the authorization of multinational clinical trials in the context of public health emergencies". We recommend extending this procedure to critical medicines and rare diseases.

Pathway for combined studies (new article 14c CTR)

EHA endorses the creation of a dedicated pathway for the authorization and conduct of combined studies, with a coordinated assessment procedure. We encourage decisionmakers to rely on the insights of the COMBINE initiative.

Use of AI in clinical trials (new article 27e)

Given the increased use of AI within clinical research, the CTR must **lay down future-proof requirements to safeguard patient safety.** The amendments on risk assessment and protocol disclosure are therefore crucial.

We welcome the development of **comprehensive CTAG guidelines** (pursuant to Article 85(5)), which should require sponsors to showcase how they will (1) prevent and address potential risks, biases and errors and (2) inform patients in an accessible manner.

Alignment with the EHDS and the GDPR

Data access under the 'secondary use' framework created by the EHDS should also benefit clinical trials. EHA therefore welcomes the amendment to Article 25.

We also welcome the changes to Article 93, to ensure alignment between the CTR and the GDPR, and a harmonized legal basis for processing data—including helpful clarifications regarding when sponsors and investigators, respectively, are required to process personal data or data concerning health.

Reducing regulatory barriers for paediatric research (Articles 10 and 32)

As recognised in the 2024 revision of the WMA Declaration of Helsinki, excluding vulnerable individuals, groups or communities with distinctive health needs from medical research can perpetuate and exacerbate disparities. The harms of exclusion should thus be weighed against the harms of inclusion. EHA fully agrees with Article 10, (new) paragraph 6, as it appropriately reflects these concerns.

Article 32(1), point e results in clinical trials for diseases affecting both children and adults to be primarily conducted with adults; hence EHA supports the removal of this regulatory barrier.

Improved efficiency in Part I (Articles 6 and 16a)

EHA welcomes the involvement of the ethics committee of the RMS in reviewing Part I of the assessment report. We also welcome moving the assessment of the translation 'adequacy' to Part II. EHA moreover supports the amendments creating a procedure for parallel substantial modification regarding aspects covered by Part I or part II (new Article 16a).

Harmonisation in Part II (Articles 7 and 25)

EHA supports the amendment to Article 7 of the CTR, allowing Member States concerned (MSCs) to rely on the ethical review conducted by the ethics committee of the RMS for the common elements of Part II. Furthermore, **EHA fully endorses the use of harmonised templates for the submission of part II documents**, which will reduce fragmentation between Member States and alleviate the administrative burden (Recital 134 and Article 25).

Electronic informed consent (articles 2 and 29)

EHA endorses a broader definition of informed consent, as foreseen in the amendments, including "consent given through the use of electronic systems, methods and processes, and signed electronically".

Alignment between ethics committees and national competent authorities (Articles 83 and 83a)

EHA welcomes the amendments to Article 83 stating that Member States should ensure that their ethics committees and national competent authorities have expertise and resources, as well as the newly introduced provision (Article 83a) to enhance communication and coordination between the two.

Clinical Trials Coordination and Advisory Group (Article 85)

The CTAG will be instrumental in ensuring the harmonised and streamlined implementation of the CTR amendments. EHA therefore welcomes a more comprehensive outline of its tasks. **Clinical experts and, where relevant, patient representatives should be systematically involved to ensure that implementation guidance is aligned with their reality and needs.**

(Biotech Act, Article 58)
Regulation No 536/2014

- **Article 85, paragraph 3**

European Commission proposal	EHA amendment
3. For the purpose of the fulfilment of their tasks, CTAG members shall be	3. For the purpose of the fulfilment of their tasks, CTAG members shall be able

able to rely on the contribution of experts from national competent authorities and ethics committees. These experts shall participate in CTAG meetings where relevant.	to rely on the contribution of experts from national competent authorities and ethics committees. These experts shall participate in CTAG meetings where relevant. ***The CTAG shall consult with scientific experts and patient representatives, where relevant.***

- **Article 85, paragraph 5, point (e)**

European Commission proposal	EHA amendment
(e) to contribute to the development of guidance aiming to ensure effective and harmonised implementation of this Regulation.	(e) to contribute***, in collaboration with scientific experts, medical societies, and healthcare professionals,*** to the development of guidance aiming to ensure effective and harmonised implementation of this Regulation.