Regulatory Brief by ECNE Research | 5 June 2026

This Week's Top Global Headlines

U.S.A

- **FDA issues draft guidance to help accelerate cell and gene therapy development:** On 2 June 2026, FDA issued draft guidance on leveraging prior knowledge in the development of human gene therapy products incorporating genome editing. The guidance is intended to help sponsors make greater use of existing scientific and regulatory knowledge, including CMC data, nonclinical results, and clinical information, to streamline development and avoid unnecessary duplication. It reflects the FDA's commitment to get safe and effective cell and gene therapies to patients faster, particularly those living with rare and life-threatening diseases who have few or no other treatment options. **More info here.**
- **FDA updates draft guidance on manufacturer communications with payors:** FDA also issued revised draft guidance on 2 June 2026 on drug and device manufacturer communications with payors, formulary committees, and similar entities. The guidance addresses healthcare economic information and communications about unapproved products or unapproved uses, reinforcing the growing importance of evidence that can support value, access, and reimbursement discussions before and after approval. **More info here.**

EU

- **EMA publishes 2025 AI Observatory report:** EMA published its 2025 AI Observatory report on 4 June 2026, alongside updates to its artificial intelligence resources (**view the AI webpage here**). The update reflects the agency's continued focus on how AI is being monitored, governed, and applied across regulatory science, medicines development, and lifecycle oversight. **More info here.**
- **EMA advances work on external controls for evidence generation:** EMA published a concept paper on 3 June 2026 on developing a reflection paper for the use of external controls in regulatory decision-making. The reflection paper will describe the main challenges with external controls and further discuss the circumstances and methodological constraints under which the use of external controls could be considered appropriate for generating pivotal or supportive evidence, either for efficacy, safety or other relevant regulatory decision-making objectives. The work is relevant for sponsors exploring how historical, real-world, or external comparator data may support regulatory evidence packages where traditional randomized controls are challenging or impractical. **More info here.**

APAC

- **Singapore's HSA highlights factors affecting COVID-19 antigen rapid test accuracy:** Singapore's Health Sciences Authority published guidance on factors that may affect the accuracy of COVID-19 antigen rapid self-test kit results. The update reminds users that test performance can be influenced by timing of testing, sample collection technique, kit storage, expiry dates, and correct interpretation of results. For diagnostics manufacturers and public-health stakeholders, it reinforces the importance of clear instructions for use, product quality, and post-market communication to support reliable testing in real-world settings. **More info here.**
- **Australia's TGA publishes Pharmacovigilance Inspection Program metrics report:** On 2 June 2026, TGA published a report summarizing outcomes from its Pharmacovigilance Inspection Program for January 2023 to December 2024. The report highlights inspection findings and case studies of critical deficiencies, supporting medicine sponsors in strengthening pharmacovigilance systems and lifecycle compliance. **More info here.**

Deep Dive: Evidence Built for the Lifecycle

This week's regulatory updates highlight not only the importance of the amount of evidence generated, but also whether that evidence is scientifically justified, reusable, governed appropriately, and fit for the decisions it needs to support.

In the U.S., FDA's draft guidance on leveraging prior knowledge in genome-editing gene therapy development signals a more strategic approach to evidence generation. For sponsors developing complex therapies, the ability to use existing CMC, nonclinical, and clinical knowledge may help reduce unnecessary duplication and accelerate development. But it also raises the bar for evidence logic. Sponsors need to show why prior knowledge is relevant, how it applies to the product under review, and where new evidence is still needed.

FDA's updated draft guidance on manufacturer communications with payors points to the same issue from a different angle. Evidence is interpreted by payors, formulary committees, health systems, and other decision-makers who are assessing value, access, and appropriate use. That means sponsors need evidence strategies that can support scientific review and economic communication without creating inconsistency or overreach.

In Europe, EMA's 2025 AI Observatory report reinforces the importance of governance in evidence generation. As AI becomes more embedded in medicines development, regulatory operations, pharmacovigilance, and lifecycle oversight, the question is not simply whether AI can accelerate work. The question is whether AI-enabled processes are transparent, reliable, explainable, and appropriately controlled.

EMA's concept paper on external controls brings this challenge into sharp focus. Historical, real-world, or external comparator data may be valuable in settings where traditional randomized controls are difficult or impractical. But these approaches also require careful attention to bias, data quality, comparability, methodology, and interpretability. The value of external controls depends not only on access to data, but on whether the data can credibly support the regulatory question being asked.

Across APAC, the same themes appear through post-market oversight and real-world use. Singapore's HSA update on COVID-19 antigen rapid test accuracy highlights the continuing role

of regulators in supporting reliable diagnostic performance after products reach users. Test accuracy can also be affected by timing, sample collection, storage, expiry, and correct interpretation of results. For diagnostics sponsors, this reinforces the importance of clear instructions for use, product quality, and effective post-market communication.

Australia's TGA pharmacovigilance inspection metrics report brings the same point into medicines oversight. Lifecycle compliance is not theoretical. Sponsors are expected to maintain pharmacovigilance systems that can detect, evaluate, manage, and learn from safety issues in practice. Together, these updates show that regulatory confidence increasingly depends on what happens after authorization i.e., whether products perform reliably, whether risks are communicated clearly, and whether sponsors have systems capable of sustaining trust in real-world use.

Taken together, this week's developments suggest that evidence strategy is becoming more integrated and more demanding. Prior knowledge, AI tools, external controls, health economic information, post-market safety systems, and public-facing product communication can all strengthen a program when they are credible, well governed, and connected to the decisions they are intended to support. For sponsors, the strongest programs will be those that can explain what evidence is being used, why it is appropriate, how it can be trusted, and how it supports the full pathway from development to access, adoption, and lifecycle confidence.

Why This Matters

Regulators and downstream decision-makers are becoming more comfortable with new types of evidence, but they are also becoming more exacting about how that evidence is justified. Prior knowledge, AI-enabled tools, external controls, real-world data, health economic information, diagnostic self-testing, and vigilance systems can all help products move more efficiently through development and into use. But their value depends on credibility, governance, transparency, and relevance to the decision being made.

For companies, this changes the evidence-planning question. It is no longer enough to ask: "What data do we need to submit?"

The better questions are:

- Can this evidence be trusted?
- Is it appropriate for this product and this decision?
- Can it support more than one stakeholder?
- Is it governed well enough to withstand scrutiny?
- Will it remain useful after authorization?

That shift matters because products increasingly move through complex pathways involving regulators, payors, HTA bodies, clinicians, procurement teams, patients, and post-market safety functions. Evidence that is not designed with those users in mind can slow review, weaken value communication, complicate adoption, or create lifecycle risk.

The opportunity for sponsors is to build evidence strategies that are both efficient and defensible. That means using prior knowledge where appropriate, applying AI and external controls with clear governance, preparing payor-facing evidence responsibly, and maintaining post-market systems that can support confidence once products reach real-world settings. The

goal is to generate evidence that can travel — evidence that is credible enough, clear enough, and connected enough to support the full pathway from development to patient access.

ECNE Insight: Build Evidence Early

The strongest evidence strategies are not built at the end of development. They are designed early, with a clear view of who will need to rely on the evidence and what decisions it must support. At ECNE Research, we see this becoming increasingly important across pharma, medtech, diagnostics, and biotech. Sponsors are under pressure to move faster, use evidence more efficiently, and communicate value more clearly. That means evidence planning cannot sit in a single function or stop at the regulatory submission. It needs to connect clinical development, regulatory strategy, health economics, diagnostics, digital tools, quality systems, vigilance, and post-market obligations.

The organisations best positioned for success will be those that can answer three questions early:

- What evidence can be reused responsibly?
- What evidence will different decision-makers trust?
- What systems are needed to sustain confidence after authorization?

This is where evidence strategy becomes a way to reduce friction, strengthen stakeholder confidence, and support the full pathway from development to patient access.

On Our Radar

- **EU | 8-11 June 2026 — EMA CTIS sponsor end-user training program:** EMA will hold its Clinical Trials Information System sponsor end-user training program online from 8–11 June 2026. The training is relevant for sponsors submitting clinical trial applications in the EEA under the Clinical Trials Regulation and for teams working to strengthen CTIS readiness, submission quality, and operational consistency. **More info here.**
- **Global | 8–10 June 2026 — WHO Prequalification Workshop for IVD Manufacturers** WHO will hold a Prequalification Workshop for manufacturers of in vitro diagnostics at its Geneva headquarters from 8–10 June 2026. The workshop will provide an overview of WHO prequalification requirements and processes, supporting manufacturers in strengthening regulatory readiness and facilitating access to quality-assured IVDs for global use. **More info here.**

The Friday Brief is curated by Elizabeth Weathers, PhD, RN, RGN, FAAN, Founder & CEO of ECNE Research. Follow ECNE Research on LinkedIn for ongoing insights in regulatory strategy, clinical evidence, and market intelligence.