Regulatory Brief by ECNE Research | 31 July 2026

This Week's Top Global Headlines

U.S.A

- **FDA advisers vote against effectiveness evidence for deramiocel in Duchenne cardiomyopathy:** On 29 July, FDA's Cellular, Tissue, and Gene Therapies Advisory Committee voted 9–3 against concluding that the available evidence established the effectiveness of Capricor Therapeutics' deramiocel for cardiomyopathy associated with Duchenne muscular dystrophy. Panel members raised concerns about changes to outcome analyses, missing information, measurement uncertainty, and whether the study population had sufficiently established cardiomyopathy at baseline. FDA is expected to make its decision by 22 August. **More info here.**
- **FDA approves a blood-based colorectal cancer screening test:** Freenome and Abbott announced FDA approval of SimpleScreen CRC, a blood-based test for colorectal cancer screening in adults aged 45 and older who are at average risk. Abbott is expected to commercialize the test in the United States this autumn. The approval expands the emerging blood-based cancer-screening category. Market adoption will depend not only on analytical and clinical performance, but on how the test fits alongside colonoscopy and stool-based screening, how positive results are managed, patient adherence, guideline inclusion, and reimbursement. **More info here.**

EU

- **EMA recommends Susvimo, a refillable ocular implant for wet age-related macular degeneration:** At its 20–23 July meeting, EMA's CHMP recommended marketing authorization for Susvimo, a ranibizumab ocular implant for adults with neovascular, or wet, age-related macular degeneration. EMA describes Susvimo as the first refillable ocular implant of its kind recommended in the EU, designed to release ranibizumab over time and be refilled every six months. The recommendation is relevant for sponsors developing products that combine medicines, delivery systems, and long-term treatment management, where clinical benefit, procedure-related risk, usability, refill logistics, and patient follow-up all shape real-world adoption. **More info here.**

APAC

- **Hansoh/GSK oncology asset meets Phase III endpoint in relapsed osteosarcoma:** GSK and China's Hansoh Pharmaceutical reported that the antibody–drug conjugate risvutatug rezetecan, or Ris-Rez, met the primary endpoint of progression-free survival in a Phase III study involving patients with osteosarcoma who had progressed or relapsed after at least two prior lines of systemic therapy. Hansoh plans to engage with China's Center for Drug Evaluation to advance preparations for a biologics application, while GSK holds rights outside China. The development reflects China's increasing

contribution to globally relevant oncology pipelines and the growing importance of evidence packages that can support confidence across jurisdictions. **More info here.**

Deep Dive: When Is Imperfect Evidence Sufficient?

This week's headlines point to one of the hardest questions in regulatory strategy: when is imperfect evidence sufficient to support the next decision?

The clearest example comes from FDA's advisory-committee review of Capricor Therapeutics' deramiocel for cardiomyopathy associated with Duchenne muscular dystrophy. The therapy addresses a serious condition with significant unmet need, but the committee voted against concluding that the available evidence established effectiveness. Panel concerns focused on changes to outcome analyses, missing information, measurement uncertainty, and whether the study population had sufficiently established cardiomyopathy at baseline.

That distinction matters. Unmet need can justify urgency, flexibility, and regulatory engagement, but it cannot replace interpretable evidence. For advanced therapies, especially in rare or serious conditions, sponsors need to show not only a promising signal, but a signal that is sufficiently reliable, clinically meaningful, and aligned with the proposed indication.

The approval of SimpleScreen CRC, Freenome and Abbott's blood-based colorectal cancer screening test, shows a different side of the same issue. Screening tests are often judged not only by analytical and clinical performance, but by how the results will be used in practice. For SimpleScreen CRC, adoption will depend on how the test fits into existing screening pathways, how positive results are managed, whether patient's complete follow-up colonoscopy, how clinicians communicate results, how guidelines respond, and whether payers see sufficient value.

EMA's recommendation of Susvimo adds another layer. A refillable ocular implant for wet age-related macular degeneration is not just a medicine story. It is also a delivery-system, procedure, follow-up, usability, and long-term treatment-management story. The clinical evidence must support the benefit-risk profile, but real-world success will also depend on refill logistics, clinician training, patient selection, procedure-related risk management, and ongoing monitoring.

The China-developed oncology asset Ris-Rez also reflects the increasing importance of evidence that can travel across jurisdictions. A positive Phase III endpoint in relapsed osteosarcoma is an important development, but global relevance will depend on whether the study population, comparator, endpoint definitions, manufacturing controls, and benefit-risk findings can support regulatory confidence beyond the original development setting.

Together, these developments show that evidence strategy has to be built around the next decision, not only the first decision.

For regulators, the question may be whether the evidence supports approval or authorization. For clinicians, it may be whether the product fits into care. For patients, it may be whether the benefits and risks are understandable. For payers, it may be whether the evidence supports value. For health systems, it may be whether the product can be implemented safely, consistently, and sustainably.

The strongest evidence strategies anticipate these questions early. They connect clinical endpoints, intended use, patient selection, usability, follow-up, reimbursement, post-market monitoring, and lifecycle evidence into one coherent plan.

This week's message is clear: imperfect evidence may be acceptable, but only when the uncertainty is understood, justified, and connected to a credible plan for what comes next.

Why this Matters

This week's headlines show that regulators, clinicians, payers, and health systems are often willing to consider new approaches where unmet need is high, access barriers are real, or existing care pathways are insufficient. But flexibility still depends on whether the evidence can be interpreted, defended, and applied to the decision at hand.

That is especially important for advanced therapies, diagnostics, combination-style delivery systems, and globally developed assets. These products often enter areas where traditional evidence models may not fit neatly, but that does not reduce the need for clarity around the intended population, endpoint selection, clinical relevance, missing data, follow-up, usability, and real-world implementation.

The practical risk for sponsors is assuming that a positive signal is enough.

A promising endpoint, an innovative delivery system, or a more accessible testing option may create momentum, but each still needs an evidence package that explains what the product does, who it is for, how it should be used, what uncertainty remains, and how that uncertainty will be managed over time.

For healthcare and life-sciences organizations, the evidence strategy should therefore be built around the full decision pathway:

- Can regulators interpret the evidence?
- Can clinicians apply it in practice?
- Can patients understand the benefits and risks?
- Can payers assess value?
- Can health systems implement the product safely and consistently?
- Can post-market evidence close remaining gaps?

The products most likely to move successfully from innovation to adoption will be those supported by evidence that is not only positive, but usable, explainable, and durable.

ECNE Insight

Too often, evidence strategies are built around the first regulatory milestone, with access, adoption, post-market requirements, and lifecycle evidence treated as later problems. This week's headlines show why that approach is becoming increasingly risky.

At ECNE Research, we see the strongest programmes as those that define the evidence pathway early. That means understanding not only what is required to support approval or authorization, but what will be needed to support clinical use, reimbursement, guideline positioning, patient follow-up, and continued confidence after launch.

That may mean:

- ensuring endpoints, missing data, analysis changes, and confirmatory plans can withstand regulatory scrutiny.

- demonstrating how the test fits into real-world screening pathways, follow-up algorithms, patient behaviors, and payer decision-making.
- connecting clinical benefit with procedure-related risk, usability, refill logistics, training, and long-term monitoring.
- anticipating whether data generated in one region can support confidence in another.

Regulators, clinicians, payers, patients, and health systems may all ask different questions, but they are often relying on the same evidence base. The sponsors that plan for this early will be better positioned to move from regulatory decision to adoption, access, and lifecycle confidence.

On Our Radar

- **EU | 2 August 2026 — EU AI Act Article 50 transparency obligations begin to apply**: Providers and deployers of applicable interactive and generative AI systems will need to comply with new disclosure, marking, and transparency requirements. This is especially relevant for healthcare and life-sciences organizations using AI across patient-facing, clinician-facing, educational, operational, or product-related workflows. **More info here.**
- **USA | 3 August 2026 — FDA comment deadline for draft guidance on communications with payors:** Comments are due by 3 August 2026 on FDA's revised draft guidance on drug and device manufacturer communications with payors, formulary committees, and similar entities. The guidance is relevant for teams developing healthcare economic information, value communication strategies, and evidence packages intended to support access and reimbursement discussions. **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.