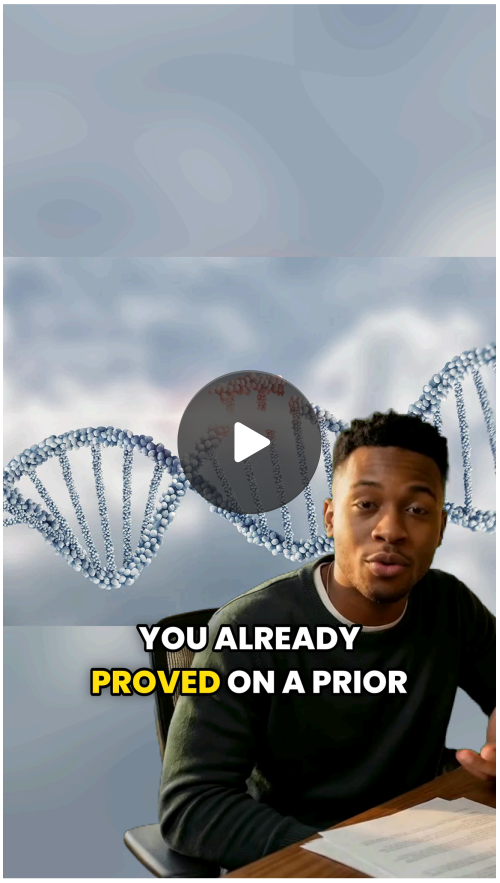
How FDA’s June 2026 Draft Guidance Lets Genome Editing Sponsors Reuse CMC, Nonclinical, Bioinformatics, and Clinical Data Across Programs, and Where the Agency Still Wants Produc Specific Work Done

JUN 06, 2026 • PAID

Video Preview



🎧 Podcast episode for paid subscriber only. Also available on Spotify.

Thoughts on Healthcare Markets & Technology

How FDA's June 2026 Draft Guidance Lets Genome Editing Sponsors Reuse CMC, Nonclinical, Bioinformatics, and Clinical Data Across Programs, and Where the Agency Still Wants Product Specific Work Done

FDA just dropped a draft guidance that every genome editing platform company should read. The question it answers: when can you stop regenerating data you already proved on a prior program...

Listen now

7 minutes ago · Thoughts on Healthcare

To listen to paid episodes in Apple or Spotify, link your Substack subscription via the s settings on those platforms (instructions inside the Substack app under Subscriptions → Podcast).

Table of Contents

Why this guidance exists and what PDUFA had to do with it

The vocabulary problem, public knowledge versus platform knowledge

CMC, where the leverage actually saves real money

Stability and comparability, the data that travels between products

Nonclinical, riding the delivery vehicle until the cargo bites back

Bioinformatics and off-target, where the guide RNA spoils the party

Clinical data, natural history studies, and the rare disease math

How you actually submit this, and what it means for the people building these companies

Abstract

FDA's Center for Biologics dropped a draft guidance in June 2026 on leveraging knowledge for human gene therapy products that incorporate genome editing, b vivo and in vivo. It is a PDUFA VII deliverable, comment only, nonbinding, and i to answer the single most expensive question in the field: when can a sponsor sto generating brand new data and instead point at something it or someone else alr proved.

Quick orientation. The guidance defines three buckets: public knowledge, platfo knowledge, and the umbrella term prior knowledge. Platform knowledge comes flavors (internal company, third-party via master files, publicly available, and consortium/data-sharing). It walks through 3 domains where reuse can happen: C (analytical methods, lot release specs, stability, comparability, process characteri and validation, facilities, cell banking), nonclinical (product-type reasoning, biodistribution, tox, genotox, DART, transgene), and clinical (trial design, clin p natural history, real-world evidence, long-term follow-up). The throughline is dependence. Anything independent of the edit itself (an analytical method, a del vehicle's biodistribution, a cleanroom classification) travels well. Anything seque specific (off-target profiles, on-target outcomes, identity, potency) mostly does no The guide RNA is the line in the sand. References worth flagging: the Jan 2024 genome editing guidance, ICH Q2(R2), Q5D, S2(R1), S5(R3), S12, USP chapters 1 and 1044, and 21 CFR 601.2 and 312.23(b). Net for builders: real CMC and nonc efficiency for platform companies, near-zero relief on the parts of the dossier tha make each product its own product.

Why this guidance exists and what PDUFA had to do with it

Continue reading this post for free, courtesy of Thoughts o
Healthcare.

Claim my free post

Or purchase a paid subscription.

← Previous