



FDA to begin releasing clinical study reports in pilot programme

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The US Food and Drug Administration (FDA) has announced it will disclose clinical study reports (CSRs) in a pilot programme to begin this month.^{1,2} Participation in the pilot will be voluntary for sponsors, include up to nine recently approved drug applications, and is limited to CSRs for the key “pivotal” trials that underpin drug approval.

Declaring that “transparency can be a powerful tool for innovation,”³ FDA commissioner Scott Gottlieb also argued the public CSRs would help streamline the FDA’s evaluation of drug applications and “create a more efficient review process.”⁴

The announcement, made on 16 January at a transparency forum led by Johns Hopkins School of Public Health, thrusts the regulator into an activity senior officials have in recent years resisted, arguing that data sharing was an activity best left to the companies that own the data, not middlemen like the FDA. Historically, the agency’s position has been that laws such as the Trade Secrets Act prohibit it from releasing trial data,⁵ and there have been only a handful of cases where the FDA has released CSRs, sometimes in response to freedom of information requests but other times only following a court order.

Arti Rai, professor of law at Duke University and director of the Center for Innovation Policy at Duke Law, said “The FDA’s reliance on a voluntary mechanism suggests a continuing level of caution regarding the implications of the Trade Secrecy Act.”

An FDA spokesperson told *The BMJ*, “The FDA will redact the CSRs for trade secrets, confidential commercial information, and personal privacy information before they are posted.”

Under the pilot programme, not all sections of the CSR will be posted. “Specifically, we’ll include the study report body, the protocol and amendments, and the statistical analysis plan for each of the participating product’s pivotal studies,” Gottlieb said.²

In Europe, sections of licensing applications, including CSRs, have been publicly accessible through the European Medicines Agency (EMA) since 2010. To date, the EMA has released millions of pages of CSRs, and since late 2016 has been making clinical reports publicly available on its website (<https://clinicaldata.ema.europa.eu>).

Transparency advocates have for years called on the FDA to follow the EMA’s lead.⁵⁻⁸

Rai said that “the example of the EMA may have spurred a measure of healthy regulatory competition—a race to the top. The FDA should be applauded for this move.”

Comparing the two regulators, a FDA spokesperson told *The BMJ*: “The FDA’s information will be available to the general public, while the EMA’s information requires a login and generally requires the viewer to be from the European Union.”

However, registration for access to CSRs through the EMA’s clinical data publication website takes minutes and is open to everyone, regardless of citizenship, who agrees to use the data for non-commercial purposes. To download CSRs the EMA does, however, require a place of address in the European Union.

It is possible that sponsors interested in the FDA’s pilot may fulfil the programme requirements simply by reposting the exact CSR that the EMA has posted on its website. In an interview, the FDA did not deny this possibility, but noted that there may be “differences in redaction.”

Anna Davis of the Johns Hopkins Clinic for Public Health Law and Policy, called the programme “an appropriate balance between patient privacy and industry concerns, on the one hand, and, on the other, the broad public health benefits of increased access to scientific data related to the safety and effectiveness of medical products.”

The FDA also announced that it would begin including clinical trials’ unique NCT number in communications about specific drugs, such as product labelling and advisory committee meeting materials. “Members of the patient, academic, and scientific communities can then use this number to follow and track clinical research from a drug’s development throughout the regulatory process.”³

Tom Jefferson, an epidemiologist who led the first Cochrane review based exclusively on CSRs and other regulatory data,⁹ said including NCT numbers in FDA documents will make “the task of identifying trials quicker and 100% accurate. It’s a major step forward for reviewers.”

But Jefferson was less enthusiastic about the FDA’s CSR pilot. “Gottlieb delivered an unclear promise announcing a pilot with an unclear purpose, let’s hope this is not another window dressing piece,” he said.

Gottlieb also announced that the FDA is conducting an internal review to determine whether and how it might go about releasing portions of some of its rejection letters—formally known as complete response letters—when that letter contains information “that could have significant public health value.”³ According to research published by the FDA, 48% of letters cite both safety and efficacy deficiencies.¹⁰

Joshua Sharfstein, professor of the practice at Johns Hopkins, has been campaigning for greater transparency at the FDA for years and led the recently published *Blueprint for Transparency at the US Food and Drug Administration*,¹¹ that was the focus of the meeting on 16 January. Sharfstein told *The BMJ* that the FDA's announcement on complete response letters is "unquestionably a step in the right direction. I hope that release of these sections soon follows, and that the agency gains the confidence to regularly disclose the key reasons that products that are not approved."

Competing interests: I am a campaigner for greater transparency of clinical trial data and personally know some people quoted in this story. I also lead a RIAT Support Center, which advocates for trial transparency, funded by the Laura and John Arnold Foundation, and the Blueprint mentioned in this story was also funded by LJAF.

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