

# FDA Week

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## **THERAPEUTIC DATA STANDARDS USE COULD BE MANDATORY IN FUTURE APPLICATIONS**

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FDA is encouraging industry to use newly crafted **therapeutic data standards** -- such as utilization of qualified biomarkers -- in submissions and could start requiring companies to use the standardized **data** elements in the future, sources said following the release of the first set of standardized **therapeutic data** elements for Alzheimer's disease that they said could make reviews and approvals faster and more efficient.

The Alzheimer's **data standards** -- released earlier this month by the Critical Path Institute and the Clinical **Data** Interchange **Standards** Consortium -- creates a **standard** set of **data** elements pharmaceutical companies and other medical researchers can use to collect patient **data** during clinical trials, rather than having each company employing their own way of scoring the same endpoints.

While general **standard data** elements exist, the Alzheimer's **data standards** mark the first time **data standards** have been developed for a specific **therapeutic** area. FDA has stated that there is a need for 55 different **therapeutic data standards** for different diseases, and prioritized them based on what types of drugs they expect to be reviewing in the near future.

Standardized **data** could help FDA reviewers evaluate applications more efficiently and quickly, possibly cutting down on review time and resources, sources said. Industry could submit study **data** using biomarkers the agency has already reviewed and agreed on, rather than requiring FDA reviewers to qualify both the tools and the **data** that are submitted, said Enrique Aviles, director of **data standards** and management at the Critical Path Institute (C-Path).

FDA is concerned that the volume and complexity of non-standardized drug-related information submitted to the agency for regulatory review is creating significant challenges to the drug center's ability to efficiently perform its critical public health mission, according to an agency spokeswoman.

"It makes the job of FDA reviewers easier and enables FDA to be more efficient and effective in their approach," Aviles said of the **data standards**.

FDA is likely to encourage use of these **therapeutic data standards** as they are released and could require them to be used in future submissions, said Brian Corrigan, senior director of the primary care business unit at Pfizer. FDA's Center for Drug Evaluation and Research "strongly encourages [investigational new drug] sponsors and [new drug application] applicants to consider the implementation and use of **data standards** for the submission of applications", according to an agency spokeswoman.

Although submission of clinical study **data** in electronic format has become relatively routine, these **data** are not often standardized, according to FDA. The agency has announced that it intends to propose a new federal regulation that would require submission of standardized electronic study **data**.

Corrigan said there is an expectation now that companies use standardized lab values, and that could extend to these new **standards** once they are developed. He said that new projects that are starting and those that will start in the next few years could be required to use the standardized **data** elements, although Corrigan said he assumes FDA will give industry fair notice of when they plan to require their use.

"My guess is that they would (make the **standards** mandatory)," he said. "They definitely support **data standards** and encourage us to use it now and my guess is that their encouragement will get a little stronger."

The **standards** -- which were developed with consensus from industry, advocacy groups and regulators -- pooled **data** from 11 recent Alzheimer's disease clinical research studies to create **standard** elements for assessing progression of the disease.

The database will enable researchers to more accurately project the course of mild cognitive impairment as it progresses to Alzheimer's disease, enabling the design of more efficient clinical trials that have the maximum chance of demonstrating whether a new treatment is safe and effective, according to C-Path. Corrigan said the **data standards** and the database will also help FDA and others conduct meta-analysis of the **data**, with the common **standards** allowing the agency and researchers to compare "apples to apples."

C-Path said pooling clinical **data** helps gain new insights and leverage the efforts of companies that are developing new therapies. It also means that information won't be lost and mistakes won't be repeated, which can happen when trials cannot be compared to one another, the groups said.

"Endpoints (for Alzheimer's disease) are highly variable," Corrigan said. "Having a **standard** to help decrease the variability in endpoints will make it easier to understand what is going on in your trials."

Aviles said the **data** has already been used to project how patients may respond to a placebo during a drug trial, which can help researchers know earlier if there is a problem with their study and avoid continuing with a trial that is not going to be successful.

"If the results that are submitted can be compared to projections ... that enables FDA to have more confidence in the results and they are able to assess the results more quickly," he said.

In the future, Aviles said the information could also be tied into electronic medical records.

Corrigan said while industry supports the new **data** elements, there is concern about the work that will be required to shift over to the new **standards** once a program is already underway. Aviles said some researchers are also concerned that the **standards** will limit their creativity.

But Corrigan said in the long run, the **standards** will make it easier for companies.

"So, doing it that first time, it is work and it takes resources for companies to do that," he said. "But at the end of the day, most people agree that having one **standard** describing the same **data** for different clinical trials is a very useful thing." -- Nanci Bompey