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COMMENTARY

21st Century Cures Act–February 11, 2017, Deadline for Company Disclosure of Expanded Access Programs

The landmark 21st Century Cures Act[1] ("Cures Act"), which was signed into law by President Obama and became effective on December 13, 2016 ("Enactment Date"), imposes a deadline by which the manufacturer or distributor of an investigational drug for the diagnosis, monitoring, or treatment of a serious disease or condition[2] must make available such company's policy for evaluating and responding to requests for investigational drug submitted to it as permitted by existing Food and Drug Administration ("FDA") expanded access rules.[3]

The deadline by which a company subject to the requirement must come into compliance is the *later* of (i) 60 calendar days after the Enactment Date; *or* (ii) the first initiation of a phase 2 or phase 3 study with respect to such investigational drug. Thus, for those companies that have already initiated a phase 2 or phase 3 study with respect to an investigational drug, the deadline for compliance is *February 11, 2017.*

Expanded access (sometimes referred to as "compassionate use" or "treatment use") is a mechanism by which FDA may approve the limited use of an investigational medical product (i.e., a drug or biologic that has not been approved by FDA) outside of a clinical trial, to patients having a serious disease or condition.[4]

Current FDA regulations allow for: (i) expanded access for individual patients, including for emergency use; (ii) intermediate-size patient populations; and (iii) widespread treatment use. Each type of expanded access is subject to certain regulatory conditions, including FDA authorization.[5]

FDA's expanded access regulations do not require a manufacturer to provide an investigational drug or biologic in response to a request for expanded access.[6] Similarly, the Cures Act's new transparency requirement also does *not* require that a company provide an investigational drug or biologic under an expanded access program. It also does not require that a company modify an existing program or policy by which it may elect to provide expanded access to an investigational drug or biologic.[7]

Disclosure of information regarding whether a company has an expanded access policy and to what extent it will consider and respond to a request for expanded use historically has been left to the companies themselves. The Cures Act changes this standard. The Cures Act requires that the expanded access policies be made public and readily available, such as by posting the policies on a publicly available internet website.[8] Moreover, the policies must include:

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- Contact information for the manufacturer or distributor;
- The procedure for making requests under the policy;
- The general criteria the manufacturer or distributor will use to evaluate and respond to requests;
- The length of time the manufacturer or distributor anticipates will be necessary to acknowledge receipt of requests; and
- A hyperlink or other reference to the clinical trial record containing information about the expanded access for such drug (as required by the Public Health Service Act[9]).[10]

A policy may be generally applicable to all investigational drugs of a company,[11] and a company may revise its policies at any time.[12]

For companies that have, to date, addressed expanded access requests on an informal basis, the new transparency requirements of the Cures Act may require prompt consideration of such companies' internal policies and practices. For companies with existing formal policies, the Cures Act may require consideration of fundamental elements of such existing policies such as explanation of the request procedures, relevant criteria, and response timing.

Lawyer Contacts

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Endnotes

1 Pub. L. No. 114-255, 130 Stat. 1033 (2016).

2 *Id.* at § 3032, 130 Stat. at 1100-1101 (to be codified at 42 U.S.C. § 21 U.S.C. § 360bbb–0).

3 *See* 21 U.S.C. § 360bbb and 21 C.F.R., Part 312, Subpart I (together, "FDA Expanded Access Rules"). *See also* "FDA Guidance for Industry: Expanded Access to Investigational Drugs for Treatment Use—Questions and Answers" (June 2016).

4 *See* 21 C.F.R. Part 312, Subpart I.

5 21 C.F.R. §§ 312.305-320.

6 21 C.F.R. Part 312.

7 This is consistent with FDA's Expanded Access Rules.

8 § 3032, 130 Stat. at 1101 (to be codified at 21 U.S.C. § 360bbb-0(b)).

9 *See* 42 U.S.C. § 282(j).

10 § 3032, 130 Stat. at 1101 (to be codified at 21 U.S.C. § 360bbb-0(c)).

11 *Id.* (to be codified at 21 U.S.C. § 360bbb-0(b)).

12 *Id.* (to be codified at 21 U.S.C. § 360bbb-0(e)).

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