From: [redacted]
To: OC GCP Questions
Subject: Questions on new expanded access regulations
Date: Wednesday, December 06, 2017 6:35:06 AM
Attachments: [redacted]

From: Morin, Steve
Sent: Tuesday, December 05, 2017 7:52 AM
To: [redacted]
Cc: [redacted]
Subject: RE: Questions on new expanded access regulations

Hi [redacted],
Thank you for your patience. We reached out to CDER Office of Medical Policy and their response is below.

This email is in response to your question concerning the *Expanded Access to Investigational Drugs for Treatment Use – Questions and Answers* guidance. You inquired about Question 25, which states, "the sponsor must report an adverse event as a suspected adverse reaction only if there is evidence to suggest a causal relationship between the drug and the adverse event", specifically, asking the following two questions:

1. Does this mean that only adverse events and serious adverse events that have sufficient evidence to support a causal relationship to the experimental drug/device need to be reported to FDA?
2. Or conversely, does this mean all events need to be reported, but are not reported under the classification of "suspected adverse reaction"?

INDs and protocols submitted for expanded access use under 21 CFR 312 Subpart I must also comply with the FDA's IND safety and annual reporting requirements, found in 21 CFR 312.32 and 312.33.

Specifically, with respect to IND Safety Reports, a sponsor must report to FDA all serious and unexpected suspected adverse reactions (312.32(c)(1)(i)). The statement you quote in your inquiry describes how FDA defines a suspected adverse reaction, i.e., any adverse event for which there is a reasonable possibility (e.g., evidence to suggest a causal relationship between the drug and the adverse event) that the drug caused the adverse event. Any suspected adverse reaction that is both serious and unexpected must be reported.

We note that sponsors of expanded access INDs or protocols are also responsible for submitting annual reports when the IND or protocol continues for 1 year or longer. As indicated in 312.33(b)(1) and (2), sponsors must submit, as part of the annual report, a narrative or tabular summary showing the most frequent and most serious adverse experiences by body system and a summary of all IND safety reports submitted during the past year.

In addition, for individual patient expanded access INDs, the licensed physician or sponsor must provide FDA with a written summary of the results of the expanded access use, including adverse effects (312.310(c)(2)).

Thank you and we hope you have a great day.
Steve

Steve L. Morin, R.N., B.S.N.
CDR, US Public Health Service
Health Programs Coordinator

Office of Health & Constituent Affairs
Office of External Affairs
U.S. Food and Drug Administration
Tel: 301-796-0161
steve.morin@fda.hhs.gov

From: [redacted]
Sent: Wednesday, November 29, 2017 4:04 PM
To: Morin, Steve <Steve.Morin@fda.hhs.gov>
Subject: RE: Questions on new expanded access regulations

Hi Steve,

Sounds great, many thanks!

Best,
[redacted]

[redacted]

From: Morin, Steve [mailto:Steve.Morin@fda.hhs.gov]
Sent: Wednesday, November 29, 2017 4:04 PM
To: [redacted]
Subject: RE: Questions on new expanded access regulations

Hi [redacted],
So the person who is able to help was just back to work today and she said if she could have a few days to verify her thinking. I will touch base with her tomorrow afternoon and then get back in touch with you.
Thank you
Steve

Steve L. Morin, R.N., B.S.N.
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FDA U.S. FOOD & DRUG ADMINISTRATION

From: [redacted]
Sent: Wednesday, November 29, 2017 11:12 AM
To: Morin, Steve <Steve.Morin@fda.hhs.gov>
Subject: RE: Questions on new expanded access regulations

Hi Steve,

I hope you enjoyed the Holiday!

Thanks again for your reply. I wanted to quickly check in and see if you have any updates on my inquiry.

Best Wishes,
[redacted]

[redacted]

[redacted]

[redacted]

From: Patient Network [mailto:PatientNetwork@fda.hhs.gov]
Sent: Wednesday, November 22, 2017 7:42 AM
To: [redacted]
Subject: RE: Questions on new expanded access regulations

Hi [redacted],
I have just reached out to a few colleagues to ensure we give you a response that is most up to date with our practice. Most people seem to be out for the holiday and I hope to have a response by next week.
Thanks
steve

Steve L. Morin, R.N., B.S.N.
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From: [redacted]
Sent: Tuesday, November 21, 2017 3:42 PM
To: Patient Network <PatientNetwork@fda.hhs.gov>
Subject: Questions on new expanded access regulations

Dear FDA,

I had a question on the revised "Expanded Access to Investigational Drugs for Treatment Use — Questions and Answers Guidance for Industry". On question 25 it states: "the sponsor must report

an adverse event as a suspected adverse reaction only if there is evidence to suggest a causal relationship between the drug and the adverse event". Does this mean that only adverse events and serious adverse events that have sufficient evidence to support a causal relationship to the experimental drug/device need to be reported to FDA? Or conversely, does this mean all events need to be reported, but are not reported under the classification of "suspected adverse reaction"?

Please let me know if my question requires further clarification.

If you prefer I would be happy to discuss this on the phone and I can be reached at (518)859-8923.

Best,

[redacted]