When FDA Says, "It Ain't Good Enough" – Rare Litigation over Pediatric Exclusivity

Deborah L. Livornese and Kalie E. Richardson

Well, the other Boss, Bruce Springsteen, not FDA, used those words. When FDA said essentially the same thing less lyrically, but with better grammar, Amgen initiated a lawsuit on May 24, 2017, over an infrequently litigated piece of food and drug law: pediatric exclusivity.

The pediatric exclusivity process was created under the Best Pharmaceuticals for Children Act (BPCA)[1] with the objective of encouraging sponsors to conduct pediatric studies and improve labeling for drugs used in children. Clinical trials are typically conducted in adults only, resulting for most drugs in a lack of data on safe and effective use in children and physicians sometimes making prescribing decisions as if children were simply small adults. Before touching on the specifics of the Amgen lawsuit, we'll review the basics of how pediatric exclusivity works.

Pediatric Exclusivity

In order for a drug to qualify for pediatric exclusivity, FDA must first issue a Written Request to the new drug application (NDA) or investigational new drug (IND) holder, describing the pediatric studies needed and a timeframe for completion. Unlike pediatric studies conducted under the Pediatric Research Equity Act (PREA), BPCA studies are voluntary. The recipient of a Written Request can complete the requested studies, or not, or it can request an amendment to revise the study requirements. Alternatively, firms can request on their own initiative that FDA issue a Witten Request.

The sponsor then submits the requested studies, typically as an NDA or supplemental NDA (sNDA). If the studies meet the specifications of the Written Request (more on what this means in the discussion of Amgen below), then exclusivity will be granted. FDA approval of pediatric labeling is not required to qualify for pediatric exclusivity. For example, if a sponsor completes the studies as requested in the Written Request, but the studies show that the product is not suitable for use in children, it would still be eligible for pediatric exclusivity based on the completion of the studies. A total of 214 pediatric exclusivities have been granted.[2]

Pediatric exclusivity is unique in that it does not run concurrent with other exclusivities, such as new chemical entity or orphan drug, or with existing patent life. Pediatric exclusivity is appended to the end of and extends any other unexpired exclusivity or listed patent life for the drug. However, because pediatric exclusivity is an *add-on*, pediatric exclusivity must be awarded at a time that the product will be covered by another type of exclusivity for at least another nine months (more on this below, too).

The Amgen Lawsuit

Amgen sought a Written Request from FDA for four different pediatric studies of its drug Sensipar[3] in May 2007. After several years of back and forth, FDA issued Amgen a Written Request in May 2010. The Written Request was subsequently amended five times. Some of the amendments were

1 21 U.S.C.A. § 355a

2 https://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/DevelopmentResources/UCM223058.pdf (last accessed June 16, 2017)

3 Sensipar is used to treat hyperparathyroidism in patients with chronic kidney disease on dialysis

triggered by the sponsor's difficulties in recruiting patients. Amgen ultimately submitted an NDA and sNDA on November 23, 2016, with a request for a pediatric exclusivity determination. On May 22, 2017, FDA denied the request for pediatric exclusivity stating that Amgen had failed to provide safety data for a large enough number of children, which FDA said was an important element of the Written Request. Three days later, Amgen filed a complaint and a motion for preliminary injunction in the U.S. District Court for the District of Columbia.[4]

In its complaint, Amgen alleges FDA acted arbitrarily and capriciously in denying the exclusivity request. FDA based its denial on Amgen's failure to fulfill just one of the criteria of the Written Request. Under the statute, FDA will accept the study reports and grant pediatric exclusivity if the submitted studies "fairly respond" to FDA's written request.[5] In its initial reply brief, FDA claims that Amgen "glosses over the fact that the study it failed to complete was a critical safety study in the youngest, most vulnerable patients."

As of June 5, the litigation is paused while Amgen seeks remedy through FDA's formal dispute resolution process. Under the judge's stipulated order, FDA's dispute resolution decision is due by August 2, 2017. The court has provided that the decision, whether made through the formal dispute resolution process or by the court, will relate back to the date of the initial determination: May 22, 2017. If FDA reconsiders its pediatric exclusivity determination then this litigation will end here, but if FDA reaffirms its denial, then we will surely see Amgen and FDA back in court.

AGG Observations

1. Given that pediatric exclusivity adds on to the end of existing exclusivity periods, those extra six months can be extremely valuable. If the Sensipar exclusivity ends up going to trial and Amgen prevails, it is likely we will see more of these cases in the future.
2. We wouldn't be surprised to see FDA determinations subject to additional scrutiny in-house at FDA going forward with the result that FDA will issue denials that have been even more extensively vetted on both the clinical review and legal side.
3. We may also see sponsors attempting to define what's "enough" up front or ask FDA to acknowledge foreseeable challenges in doing the studies.

4 *Amgen Inc. v. Price*, 17:cv-01006 (D.D.C. May 24, 2017)
5 21 U.S.C.A. § 355a(d)(3)

Authors and Contributors

Deborah L. Livornese
Partner, DC Office
202.677.4922
deborah.livornese@agg.com

Kalie E. Richardson
Associate, DC Office
202.677.4918
kalie.richardson@agg.com

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Atlanta Office
171 17th Street, NW
Suite 2100
Atlanta, GA 30363

Washington, DC Office
1775 Pennsylvania Avenue, NW
Suite 1000
Washington, DC 20006

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