



## Advisory Regarding Long-Acting Growth Hormone Medication October 2023

In June 2023 Pfizer announced they've developed a once-weekly growth hormone injection, Ngenla, expected to be available for prescribing in August 2023. Because we knew that families will want to know more about this new drug, we reached out to our PWS endocrine specialists to ask a few questions:

1. What are the potential downsides we should be on the lookout for if using a once-weekly injection versus an every-night injection?
2. Do you believe the once-weekly injection will be as effective as the once-nightly injection?
3. Might the once-weekly injection be more painful than the once-nightly injection (without considering each patient's potential higher tolerance to pain)?
4. This new medicine is approved by the FDA for use in children. Might it also be effective for use by adults (and others whose growth plates have closed)?

**The responses we received expressed great concern about the safety of *any* long-acting growth hormone injection.** Below are excerpts shared by two of our PWS Endocrine Specialists. PWCF encourages you to consult with your child's treating endocrinologist and share this information with them before you switch to a long-acting, once-weekly growth hormone injection.

**PWS Endocrine Specialist:** *"I have very serious safety concerns about these preparations. The concerns relate to prolonged exposure to high levels of growth hormone (not IGF-I levels) with these preparations. Daily growth hormone (GH) injections result in only a few minutes of elevated serum GH levels after each dose, whereas the weekly preparations give sustained high levels, raising concerns with regard to the effects of IGF-independent actions of GH... In my own practice, I will not be prescribing these preparations unless they can be shown to be safe via appropriate pre-clinical studies and long-term post marketing surveillance is implemented."*

**PWS Endocrine Specialist:** *"Another thought not related to potential safety issues is that the current formulation for long-acting GH is only approved for GH deficiency. For many patients with PWS, this is a diagnostic bar that we have not had to achieve to utilize GH and therefore may make it difficult (if not impossible) to prescribe even without the concerns raised. As GH is not approved for PWS after growth is completed, adults with PWS do generally have to have a diagnosis of GH deficiency and therefore would qualify for this and the other long-acting preparations that have recent come on the market. Long-Acting GH is not yet approved for PWS. While some families may be able to obtain it, this is not likely to be the case for many and should not be the expectation. I would generally say that use of this and other long-acting formulations is going to be a complex issue to resolve for the PWS community on several fronts."*

**For more information about Prader-Willi syndrome or for support contact PWCF  
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