# Questions regarding the proposed license to OcQuila Therapeutics, 84 FR 65169

**Rohrbaugh, Mark (NIH/OD) [E]** <rohrbaum@od.nih.gov> Tue, Jan 7, 2020 at 10:38 AM
To: kathryn ardizzone <kathryn.ardizzone@keionline.org>

I suggest not answering

Sent from my iPhone

On Jan 7, 2020, at 10:35 AM, kathryn ardizzone <kathryn.ardizzone@keionline.org> wrote:

> Dear Mr. Shmilovich:
>
> Thank you for your prompt response.
>
> I was hoping you could clarify your answers to Questions 3 and 4.
>
> Question 3 asked how the NIH fulfilled its statutory mandate to determine that exclusivity was a necessary incentive. Your answer cited the target disease and its prevalence, which you state is available online. KEI has researched XLRS and is aware of its low prevalence. I understand your response to mean that NIH considered only those factors when deciding to grant exclusivity. **Please let me know if that understanding is incorrect and what, if any, other factors were considered.** Also, please note that the license as described in the FR is not limited to XLRS. The second invention covered by the license is described as "Potentially curative therapy for XLRS**, retinoschisis, age-related macular degeneration, diabetic retinopathy, Leber congenital amaurosis, retinal detachment, cysts, cystoid, macular edema, retinitis pigmentosa, and senile schisis" and the field of use described in the FR is not limited to XLRS - it extends to "schisis cavity associated ocular disease or injury."** How does your answer account for the other disease indications?
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> Per Question 4, you referred KEI to Dr. Rohrbaugh's statements in his November 26, 2019 letter which refer to **two other licenses**. Please note that Question 4 is specific to the NIH's analysis of **this license**. Also, you call this technology "early stage." Please clarify how you define early stage. According to the PhRMA, typical drug development proceeds through four phases: (1) basic research, (2) preclinical trials, (3) clinical trials, and (4) FDA New Drug Application (NDA) filing and approval. As you know, this invention has proceeded past basic discovery and preclinical trials, and based on preclinical trial results, an IND was submitted and the invention proceeded to a **Phase I/IIa clinical trial** that has already reported some positive preliminary results. How does this meet the definition of early stage?
>
> **Finally, why is the analysis the same for this invention as those covered by the Nov. 26 letter? As far as I understand, the inventions discussed in the letter had not been investigated in human clinical trials at the time the license was noticed, so they had a different stage of development.**
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> Thank you in advance for your consideration.
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> On Tue, Jan 7, 2020 at 8:58 AM Shmilovich, Michael (NIH/NHLBI) [E] <michael.shmilovich@nih.gov> wrote:
>
> > Dear Ms. Ardizzone:

Regarding your email. Our responses are shown below in blue:

Dear Mr. Shmilovich:

Thank you for answering my colleague Luis Abinader's questions regarding the proposed license to OcQuila. I have a few questions about the licensed inventions.

1. The clinical trial NCT02317887, Study of RS1 Ocular Gene Transfer for X-linked Retinoschisis, investigated the first invention listed in the notice. **Will the second invention, Newly Improved Method and Composition for Treating Genetically Linked Diseases of the Eye, be investigated in any clinical trials, including NCT02317887?** So far, it appears that it has only been studied in mice, yet the development stage for the invention is listed as "clinical" in this licensing opportunity notice.

That is not known at this time and will be up to the licensee and NEI.

2. Can you provide us a copy of the unpublished patent applications associated with the second invention? This is not confidential business material and will help us to evaluate the license.

The PCT application has not yet published yet. Under our policy, until a PCT application is published, it is only available under a Confidential Disclosure Agreement.

3. You told Mr. Abinader that NIH is not required to perform an economic analysis to determine that an exclusive license is appropriate. **What analysis, if any, did you undergo before deciding to propose an exclusive license? If you determined that exclusivity was necessary, on what basis did you so conclude?**

XLRS is a rare disease and information about its incidence is readily available.

4. Dr. Mark Rohrbaugh, Special Advisor for Technology Transfer to the NIH Deputy Director for Intramural Research, has publicly stated that **"[t]he closer a technology is to the marketplace, the lower the risk and cost to the licensee, and the more valuable the technology from a royalty standpoint."** Mark L. Rohrbaugh, NIH: Moving Research from the Bench to the Bedside, Testimony before the House Committee on Energy and Commerce, Subcommittee on Health, July 10, 2003, https://www.govinfo.gov/content/pkg/CHRG-108hhrg88429/html/CHRG-108hhrg88429.htm. **How is the NIH negotiating this license in a way that reflects that commercial potential of these**

**inventions? Are all inventions treated equally per NIH's licensing practices regardless of development stage, risk, and cost?**

The value of patent commercialization licenses are not uniform and depend on many factors including the state of development. The present invention is early stage. Negotiation of a license, including royalties, does not occur until a final decision is made based on any competing applications and comments submitted during the notice period. The question has also been previously answered in Dr. Rohrbaugh's November 26, 2019 letter (enclosed), and the answer in that letter applies to the current case as well.

Regards,

**Michael A. Shmilovich, Esq., CLP**

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