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Re: Prospective Grant of an Exclusive Patent License: Delivery of a Corrective Glucose-6-Phosphatase-Alpha Gene to Treat Glycogen Storage Disease Type 1a (GSD-Ia) in Humans (86 FR 33321) to Panacea Opportunity, Ltd.

Dear Dr. Hubbs:

Knowledge Ecology International (KEI) would like to offer the following comments regarding the "Prospective Grant of an Exclusive Patent License: Delivery of a Corrective Glucose-6-Phosphatase-Alpha Gene to Treat Glycogen Storage Disease Type 1a (GSD-Ia) in Humans" (86 FR 33321). The license is to be granted to Panacea Opportunity, Ltd. (Panacea).

The geographic scope of the license is worldwide, and the field of use conveys the rights to the "delivery of a corrective glucose-6-phosphatase-alpha gene to treat glycogen storage disease type 1a (GSD-Ia) in humans."

We note that Panacea Opportunity, Ltd. appears similar to Panacea Opportunity Fund I, L.P., a firm that filed a "Notice of Exempt Offering of Securities" with the US Securities and Exchange Commission (SEC) on June 29, 2021, and which has as its address a P.O. Box in the Cayman Islands. The principal of the organization appears to be James Zuie-chin Huang, who is affiliated with a large number of firms and investment funds, including Ziopharm Oncology, Inc., a company the NIH sought to provide an exclusive license in January 2021. Mr. Huang and the company also seem to be related to Panacea Venture, and Panacea Venture Healthcare Fund I, L.P., which list addresses in Shanghai, China in some SEC filings, and a P.O. Box in Grand Cayman, Cayman Islands in other filings.

Glycogen storage disease type 1a is a rare disease, and occurs in approximately 1 in 100,000 births. While this is a rare disease, the NIH has an obligation to ensure that the benefits of the inventions are available to the public on reasonable terms.

The treatment will qualify for seven years of orphan exclusivity, as well as another twelve years for exclusive rights in the registration test data. Because Glycogen Storage Disease Type 1a affects infants, Panacea will also qualify for a rare pediatric disease priority review voucher, a transferable asset that has recently been sold for $100 million.

An exclusive license by the NIH must only provide the incentives reasonably necessary to bring a technology to market, according to 35 U.S.C. § 209. If the NIH has provided financial support or has otherwise de-risked aspects of the research and development of this technology and if the technology stands to qualify for further valuable incentives, the license terms should reflect this with reduced scope of duration, geographic applicability, or other terms. This is particularly important given that this firm seems to be an investment vehicle for offshore investors of unknown identities and motives.

All licenses in NIH-owned patents must include terms requiring that the resultant product is "available to the public on reasonable terms" (35 U.S.C. §§ 209(d)(3); 201(f)).

The NIH notice did not describe the state of development of any products or services relevant to the inventions. For example, we don't know if the NIH has financed or conducted any trials relevant to the licensed intellectual property. That type of information is needed to evaluate the need for an exclusive patent license, given other non-patent IP and incentives available to a developer, or how narrow or broad the scope of rights, including the years of exclusivity.

Below KEI has outlined terms that should be included to ensure transparency, protect against price discrimination, and ensure availability of any treatment in low- and middle-income countries (at affordable prices). KEI urges the NIH to include the following provisions in the terms of this license.

Prohibition against prices that discriminate against US residents

Any license should ensure that U.S. residents are not asked to pay prices that exceed the median price from the seven economies of the largest GDP and at least 50 percent of U.S. per capita income. The per capita income can be based upon the World Bank's Atlas method.

Transparency

Transparency of R&D outlays. The licensees should be required to file an annual report to the NIH, available to the public, on the research and development (R&D) costs associated with the development of any product or service that uses the inventions, including reporting separately and individually the outlays on each clinical trial. We note that this is not a request to see a company business plan or license application. We are asking that going forward Panacea be required to report on actual R&D outlays to develop the subject inventions. Reporting on actual R&D outlays is important for determining if the NIH is meeting the requirements of 35 U.S.C. § 209, that "the proposed scope of exclusivity is not greater than reasonably necessary to provide

the incentive for bringing the invention to practical application[.]" Specifically, having data on actual R&D outlays on each clinical trial used to obtain FDA approval provides evidence that is highly relevant to estimating the risk adjusted costs of bringing NIH licensed inventions to practical application.

Acknowledgement of federal funding - publication and publicity. The licensee should be required to include, when issuing statements, press releases, and other documents describing the development of any product that includes the licensed inventions, a statement that describes the role of the licensed inventions and the total and proportionate contribution of federal funding to the research and development performed to bring the inventions to market.

Additional transparency issues. The license should have provisions that give effect to the transparency norms set out in WHA72.8 "Improving the transparency of markets for medicines, vaccines, and other health products", a resolution enthusiastically supported by HHS in 2019.

Additional Provisions to Protect the Public Interest

Global registration and affordability. The licenses should require the licensee to disclose the steps that each will take to enable the timely registration and availability of the medical technology at an affordable price in the United States and in every country with a demonstrated need, according to the Centers for Disease Control and Prevention (CDC) and/or the World Health Organization (WHO), either by supplying a country directly at an affordable, publicly disclosed price and with sufficient quantities, or by providing technology transfer and rights to all intellectual property necessary for third parties to do so.

Low- and middle-income countries. The NIH should ensure that the exclusive license does not extend to countries with a per capita income less than 30 percent of the United States, in order to ensure that the patents do not lead to restricted and unequal access in developing countries. If the NIH rejects this suggestion, it needs to provide something that will give effect to the policy objective in the "United States Public Health Service Technology Transfer Policy Manual, Chapter No. 300, PHS Licensing Policy," which states the following: "PHS seeks to promote commercial development of inventions in a way that provides broad accessibility for developing countries."

Medicines Patent Pool. The NIH should retain a right to grant the WHO, the Medicines Patent Pool or other governments the rights to use the patent rights to procure the medical technology from competitive suppliers, including technology transfer, in LMICs, upon a finding by HHS or the WHO that people in these markets do not have sufficient access to the medical technology.

Conclusion

Please notify us if and when a license is granted, so we can request a copy under the Freedom of Information Act.

Sincerely,
Claire Cassedy
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Knowledge Ecology International