



Precision Health Economics Experts Share Insights with PPTA

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Changes in drug pricing and policy can be confusing at best. We went to the experts to help put into context their potential effects on plasma protein therapies.



Dr. Anupam B. Jena is the Ruth L. Newhouse associate professor of health care policy at Harvard Medical School, a physician in the Department of Medicine at Massachusetts General Hospital, and a faculty research fellow at the National Bureau of Economic Research. His research involves several areas of health economics and policy, including the economics of health care productivity and medical innovation.



Ms. Jacquelyn W. Chou is the senior director of policy & economics at Precision Health Economics. She has extensive experience in policy-facing research in a diversity of therapeutic areas, cost-effectiveness research, and

literature-based economic and epidemiological models demonstrating the benefit of innovative treatments to society.

Q What are some of the current and upcoming trends you see policymakers proposing to curb drug spending? What do you expect the impact to be for individuals who rely on plasma protein therapies?

We've seen two recent primary trends in the marketplace. First, federal policymakers have been considering a number of proposals to exert pricing controls or downward pricing pressures on manufacturers. These policies range from importation of small molecule drugs, which is less of an issue for plasma protein therapies, to international reference pricing and modifying the Medicare Part B payment system, both of which could directly impact

plasma protein therapies. Second, at a local level we have seen public and private payers increasingly rely on utilization management strategies to reduce use of treatments. The same payers are also using cost-effectiveness information in negotiations over net drug prices. Federal pricing pressure may affect individuals who use plasma protein therapies in several ways. First, the intended goal is to reduce out-of-pocket costs, which would benefit consumers. But there is a trade-off to both current and future patients if these changes in reimbursement policy adversely impact downstream innovation, which studies suggest is a legitimate concern. Plasma protein therapies are challenging to develop and manufacture. The impact of the utilization management may be more concerning for individuals who use plasma therapies. Given the uniqueness of plasma protein therapies and the variation in how each person responds to treatment, even those in the same class, limiting access to therapies could result in reduced access to optimal therapies for a given individual patient.

Q Has reference pricing or parallel pricing been effective in markets with limited capacity?

Reference pricing has been used in other markets. But, what does effective mean? Reference pricing can certainly be effective in controlling prices. But what if the measure of success is access to innovative treatment? Or life-year extension? Or improved quality of life? Further research into determining the effect of reference pricing on those types of metrics will be important to determine the effectiveness of reference pricing for a population.

Q Some U.S. proposals want to achieve the prices of countries that use traditional cost-effectiveness assessments. Are these frameworks appropriate for all medicines, including plasma protein therapies?

Traditional cost-effectiveness assessments are meant to be tools to translate clinical outcomes into economic outcomes and are a helpful starting point for assessing the benefit and cost trade-off for treatments. While important, however, these approaches miss a number of aspects that patients using plasma protein therapies may value, such as productivity gains or spillover benefits to caregivers. Cost-effectiveness analyses are constructed to answer a specific and narrow question, which may miss a number of these aspects of value. This may be particularly true for plasma protein therapies where the diseases can be chronic with high impact on daily lives of the individual, but there may not be significant life extension, which is a primary driver of gains in a cost-effectiveness analysis.

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Q How can manufacturers of rare therapies with such a unique starting material demonstrate value to policymakers?

In therapies that treat rare diseases, it is critically important for manufacturers and end-users to collaborate to ensure that patient-centered care and policies can be developed. One step in achieving this objective is high-quality qualitative research eliciting patient preferences and quantifying the value individuals with rare diseases place on various aspects of care and characteristics of treatment. Another step in achieving this objective is real-world data analysis to describe the impact and benefit of plasma protein therapies as well as to identify areas of high cost for individuals with diseases requiring plasma protein therapies and potential areas where treatment can help produce cost savings. The challenge for being able to conduct real-world data analysis for plasma protein therapies is often sample size. Manufacturers who are interested in making an investment and commitment to generating real-world data in support of these treatments should consider building and maintaining registries.

Q What impact could you see to innovation of plasma proteins if the U.S. orphan drug tax credit is eliminated?

There are several studies that suggest the U.S. Orphan Drug Act significantly incentivized innovation into rare diseases. Eliminating the orphan drug tax credit may result in payers restricting access, limiting the potential market for the manufacturer. This would likely result in fewer plasma protein therapy innovations over time as some manufacturers would conclude the potential return on investment into research and development of a plasma protein therapy is not sufficient to make those investments. Given the severe burden and immense unmet need that often characterize diseases treated by orphan drugs, reduced innovation would have a major impact on the daily lives of thousands of individuals in the United States and globally. ●