Marc Yale, Advocacy and Research Coordinator

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MACPAC: Marc has been unmuted. Marc, you can make your comment.

MR. YALE: Thank you so much for allowing me to make a comment today. My name is Mark Yale and I'm the Advocacy and Research Coordinator for the International Pemphigus & Pemphigoid foundation, and I also serve on the board of directors for Haystack Project.

Through my own experience as a rare disease patient living with pemphigoid, as well as my involvement in advocacy for pemphigus and pemphigoid patients, and others living with extremely rare conditions, through Haystack Project, I can tell you that accessing treatment can be an immense undertaking. Pemphigus and pemphigoid have been treated off-label with a variety of different therapies that have varying degrees of success in helping control our disease, and payers can make getting access to off-label treatments extremely challenging.

We are now one of the handful of ultra-rare conditions with an FDA-approved treatment, Rituxan, now considered first-line therapy for pemphigus vulgaris. There is no question that manufacturer incentives, like the FDA priority review, breakthrough therapy designation, and orphan drug designation were really vital to getting this therapy as quickly as possible to patients with pemphigus who struggle every day to control their symptoms.

We were fortunate that Rituxan has been on the market since its approval in 1997, and that due to the nature of our condition, clinical trials could be completed without surrogate endpoints. The proposal to create an additional rebate for accelerated approval, though, could be a significant deterrent to developing a new product for pemphigus.

The proposal also raises questions that do not have good answers from a policy perspective. For example, if Rituxan's pemphigus indication were achieved through accelerated approval, would an additional rebate apply, and would it only apply to that indication? It would seem that the proposal would decrease research rather than encourage post-market studies, especially when medically accepted off-label uses would be covered without the additional rebate.

Placing a disincentive on the pathway that is designed to encourage early access to promising treatments could have the greatest impact on ultra-rare disease patients like myself, and your proposal today is basically a vote against investment in patients like me.

Thank you again for your time, and I hope you consider my comments.

CHAIR BELLA: Thank you, Marc, and thank you for sharing your personal story.