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Dear House of Representatives Standing Committee on Health, Aged Care and Sport:

I am writing you about accelerating and improving access pathways to new medicines for rare diseases in Australia. I am CEO of a US-based biopharmaceutical company focused on rare liver disease. We have been actively conducting clinical research in rare disease in Australia and engaged with Australian patient advocates for our two lead indications, Alagille syndrome and progressive familial intrahepatic cholestasis (PFIC). These are two devastating genetic diseases that drive progression to liver transplant or death in the majority of afflicted children by adulthood.
As a small company, we lack the resources to pursue approval for our products across all geographies and thus I am writing to provide thoughts on the below initiative.

> *Without compromising the assessment of safety, quality, efficacy or cost-effectiveness, whether the approval process for new drugs and medical technologies, could be made more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment.*

Given our limited resources, we are forced to prioritize and sequence the regulatory interactions for our lead program. We are in the process of submitting for regulatory approval for treatment of pruritus due to Alagille syndrome in the United States and plan to submit for approval in Europe for treatment of PFIC2 by the end of the year. If pathways were established to leverage the registration and reimbursement processes of these regions, we could greatly accelerate our plans to make maralixibat widely available for patients facing these terrible conditions in Australia.

I urge you to consider the benefit of harmonizing regulatory processes in Australia with those already established in other regions in an effort to find the most expeditious route to helping patients in Australia gain access to new medications to treat rare, life-threatening conditions.

Thank you for your consideration and partnership with the patient communities to advance the treatment of rare disease.

Best regards,

[redacted]

Chris Peetz
President & CEO
Mirum Pharmaceuticals