Michelle Davis, Executive Director
MACPAC April 2021

MACPAC: Michelle, you have been unmuted to make your comment.

MS. DAVIS: Hello. My name is Michelle Davis. I joined the International Fibrodysplasia Ossificans Progressiva Association, also known as the IFOPA, in 2016 as its executive director. I'm also the mom to a son with 21 an ultra-rare cancer, synovial sarcoma.

I'm here today to talk to you mainly about FOP, and the impact on the drugs that are being developed, that this policy potentially has. FOP is an extremely rare condition affecting only one in two million people. It is one of the rarest, most disabling genetic conditions known to medicine. An FOP bone forms in muscles, tendons, ligaments, and other connective tissues, and ridges of extra bone develop across joints, progressively restricting movement and forming a second skeleton that imprisons the body in bone.

Babies with this condition will show malformation of the great toes at birth, and the progression rate is unpredictable but appears in a pattern of upper body in childhood and lower body in adolescence. Flareups can occur spontaneously or follow physical trauma such as childhood immunizations, falls, surgery, biopsy, or viral illness. No treatment exists for FOP, but parents do have hope that one of the products in clinical trials will receive approval.

Given the seriousness and rarity of FOP, investigational treatments are eligible for FDA fast-track designation as well as FDA breakthrough therapy and rare pediatric disease designations. Fast-track designation allows companies to have more engagement opportunities with the FDA and allows them to be eligible for accelerated approval and priority review, all of which will help treatments reach patients faster, especially those with ultra-rare, life-limiting, disabling, life-shortening diseases like FOP.

Please do not disincentivize the development of therapies for rare and ultra-rare disorders like FOP by penalizing an approval pathway that could be the tipping point between companies pursuing and not pursuing research.

We appreciate the need for ensuring Phase 4 post-marketing studies are completed. Our patients will benefit from the information on that more than anyone. We are also on the front line as really rare patients and can tell you how difficult it is to do those studies, how to keep those patients engaged, which is something not always in a company’s control. Maybe we should incentivize patients rather than penalize the company.

We need to have more creative than the proposal you are considering today. We need all the incentives we have to date and more in getting companies to study conditions like FOP.

Thank you.

CHAIR BELLA: Thank you.