



ALD Connect
Externally-Led Patient-Focused Drug Development Meeting
July 22, 2022
10AM-3PM Eastern
www.aldconnect.org/pfdd

What is an Externally-Led Patient-Focused Drug Development (EL-PFDD) Meeting?

The Externally-Led Patient-Focused Drug Development meeting is an opportunity to inform the FDA about what it is like to live with a particular disease. During an EL-PFDD, the FDA becomes informed of the side effects and risks patients may be willing to accept to gain a certain level of symptom relief or slowing of their disease progression. The FDA learns about patients' needs regarding new drugs, and what their preferences are for clinical trials for their disease. EL-PFDD meetings assist the FDA in knowing if a new drug addresses patient needs.

Drug sponsors also gain insight into the major concerns of patients. This helps the companies develop treatments and design clinical trials that match patients' needs and preferences. Drug sponsors learn which disease symptoms or treatment side effects are, or are not, tolerable by the patients. This helps the companies develop drugs that matter to patients. With knowledge gained from EL-PFDD Meetings, pharmaceutical companies receive advice from the FDA on developing potential drugs and therefore the meetings help to advance medicines that meet patients' needs.

Why is ALD Connect hosting an EL-PFDD?

ALD Connect believes there is an unmet need for treatments of Adrenoleukodystrophy (ALD), and in particular, a lack of treatments targeted for adult manifestations of ALD: adrenomyeloneuropathy (AMN), adult cerebral ALD, and symptomatic women with ALD. ALD Connect was granted an opportunity to present an EL-PFDD focused on ALD in adulthood with the goal of educating the FDA on what it is like to live with the adult forms of ALD: what symptoms patients experience, how their lives are impacted, what kinds of treatments they need, and what kinds of risks they willing to accept to see improvements.

Who can attend the meeting?

The meeting is free and open to the public. No registration is required.

How can I watch the EL-PFDD?

The EL-PFDD will occur virtually. On Friday, July 22, 2022 the live-stream of the meeting will be available on the ALD Connect website at www.aldconnect.org/pfdd. On the day of the meeting (July 22nd, 2022) at 10 AM Eastern, anyone can click on the link to access a livestream of the event. The event will be livestreamed in both English and Spanish. Afterwards, a recording of the event will be available on [ALD Connect's website](#) and [YouTube channel](#).

How do I participate in the EL-PFDD?

Adults with ALD and caregivers are strongly encouraged to attend and participate during the virtual EL-PFDD. On the day of the meeting, there will be an opportunity to participate in live polling and either call in or write in to share your input. Comments can also be submitted prior to the meeting using the form [on this page](#) or by emailing pfdd@aldconnect.org. The meeting will eventually be summarized as a "Voice of the Patient" report, which will be publicly available.

What happens after the meeting?

ALD Connect will write the "Voice of the Patient" report. This will be sent to the FDA and will be used to help in their decisions to approve potential new treatments for ALD. The Voice of the Patient Report will be publicly posted on ALD Connect's website.