



2024 CTX SCIENTIFIC & FAMILY MEETING
FRIDAY, JUNE 28, 2024
EAGLEWOOD RESORT & SPA, ITASCA, IL

AGENDA

11:30 - 1:00 pm	Group Lunch at Designated Tables	Burhams Dining Room
1:00 - 1:25 pm	The importance of clinical trials for drug safety, efficacy and approval/potential future clinical trials for CTX.	Dr. Florian Eichler
1:25 - 1:50 pm	Potential gene therapy for CTX; data from a mouse CYP27A1 KO model.	Dr. Gloria González Aseguinol (remote)
1:50 - 2:15 pm	Inhibitors of CYP46A1/CYP27A1 based on in vitro enzyme assays (and implications for CTX patients.	Dr. Irina Pikuleva
2:15 - 2:40 pm	Variant of FGF19 to treat cholestatic liver diseases; potential utility for bile acid synthesis disorders.	Dr. Lei Ling
2:40 - 2:50 pm	Break	
2:50 - 3:15 pm	Cholesterol accelerates α -synuclein aggregation and spreading by activating asparagine endopeptidase.	Dr. Zhentao Zhang (remote)
3:15 - 3:40 pm	Real-world data supporting use of CDCA in pregnancy in CTX.	Dr. Tzipora Falik-Zaccai
3:40 - 4:05 pm	Experience with co-administration of cholic acid and chenodeoxycholic acid.	Dr. Kenneth Setchell
4:05 - 4:30 pm	Update on RESTORE clinical trial supporting use of CDCA to treat CTX.	Dr. Austin Larson

