

Supplementary Table S1. Interventional clinical trials with translation readthrough-inducing drugs (TRIDs).

NCT Number	Title	Status	Conditions	Interventions	Phases	Start Date	Completion Date
NCT05448755	A Study of ELX-02 in Patients With Alport Syndrome	Recruiting	Alport Syndrome	Drug: ELX-02	Phase 2	November 28, 2022	May 30, 2023
NCT01141075	Ataluren for Nonsense Mutation Methylmalonic Acidemia	Terminated	Amino Acid Metabolism, Inborn Errors	Drug: Ataluren	Phase 2	July 19, 2010	November 3, 2011
NCT04117880	A Phase 2 Open Label Extension Study in Participants With Nonsense Mutation Aniridia	Withdrawn	Aniridia	Drug: Ataluren	Phase 2	December 31, 2018	January 31, 2021
NCT02647359	Study of Ataluren in Participants With Nonsense Mutation Aniridia	Completed	Aniridia	Drug: Ataluren Drug: Placebo	Phase 2	January 31, 2016	January 22, 2021
NCT04014530	Pembrolizumab With Ataluren in Patients With Metastatic pMMR and dMMR Colorectal Carcinoma or Metastatic dMMR Endometrial Carcinoma: the ATAPEMBRO Study	Recruiting	Colorectal Cancer Endometrium Cancer	Drug: Ataluren + Pembrolizumab	Phase 1 Phase 2	August 1, 2019	August 1, 2023
NCT02107859	Study of Ataluren (PTC124) in Cystic Fibrosis	Terminated	Cystic Fibrosis	Drug: Ataluren	Phase 3	May 23, 2014	June 5, 2017
NCT02456103	Extension Study of Ataluren in Participants With Nonsense Mutation Cystic Fibrosis	Terminated	Cystic Fibrosis	Drug: Ataluren	Phase 3	August 31, 2015	June 2, 2017
NCT01140451	Extension Study of Ataluren (PTC124) in Cystic Fibrosis	Completed	Cystic Fibrosis	Drug: Ataluren	Phase 3	August 12, 2010	December 2, 2013

NCT03256968	PTC Study to Evaluate Ataluren in Combination With Ivacaftor	Completed	Cystic Fibrosis	Drug: Ataluren	Phase 4	January 27, 2017	December 31, 2018
NCT00458341	A Study of Ataluren in Pediatric Participants With Cystic Fibrosis	Completed	Cystic Fibrosis	Drug: Ataluren	Phase 2	March 23, 2007	February 29, 2008
NCT00237380	Safety and Efficacy of Ataluren (PTC124) for Cystic Fibrosis	Completed	Cystic Fibrosis	Drug: Ataluren	Phase 2	November 30, 2005	May 31, 2006
NCT02139306	Study of Ataluren in Nonsense Mutation Cystic Fibrosis (ACT CF)	Completed	Cystic Fibrosis	Drug: Ataluren (PTC124) Drug: Placebo	Phase 3	August 1, 2014	November 1, 2016
NCT00803205	Study of Ataluren (PTC124) in Cystic Fibrosis	Completed	Cystic Fibrosis	Drug: Ataluren Drug: Placebo	Phase 3	September 8, 2009	November 12, 2011
NCT04126473	A Phase 2 Study to Evaluate the Safety, Tolerability, PK and PD in Cystic Fibrosis Patients With at Least 1 G542X Allele	Recruiting	Cystic Fibrosis	Drug: ELX-02 Drug: Ivacaftor	Phase 2	November 5, 2019	July 31, 2022
NCT04135495	A Phase 2 Study to Evaluate the Safety, Tolerability, PK and PD of ELX-02 in Cystic Fibrosis Patients With G542X Allele	Completed	Cystic Fibrosis	Drug: ELX-02 Drug: Ivacaftor	Phase 2	November 25, 2019	October 3, 2022
NCT00376428	Interest of Gentamicin-induced Readthrough in Cystic Fibrosis Patients	Terminated	Cystic Fibrosis	Drug: Gentamicin	Phase 2	January 2003	June 2005
NCT03256799	Evaluation of Ivacaftor in Patients Using Ataluren for Nonsense Mutations	Completed	Cystic Fibrosis	Drug: Ivacaftor/Ataluren	Phase 4	March 17, 2017	February 16, 2018

NCT00351078	PTC124 for the Treatment of Cystic Fibrosis	Completed	Cystic Fibrosis	Drug: PTC124	Phase 2	December 2006	July 2007
NCT00234663	PTC124 for Cystic Fibrosis	Completed	Cystic Fibrosis	Drug: PTC124	Phase 2	September 1, 2005	August 1, 2006
NCT00005574	Gentamicin Treatment of Muscular Dystrophy	Completed	Duchenne Muscular Dystrophy	Drug: Gentamicin	Phase 1	February 2000	January 2001
NCT03648827	A Study to Assess Dystrophin Levels in Participants With Nonsense Mutation Duchenne Muscular Dystrophy (nmDMD)	Completed	Duchenne Muscular Dystrophy	Drug: Ataluren	Phase 2	December 21, 2018	October 23, 2020
NCT03796637	A Study to Assess Dystrophin Levels in Participants With Nonsense Mutation Duchenne Muscular Dystrophy (nmDMD) Who Have Been Treated With Ataluren	Completed	Duchenne Muscular Dystrophy	Drug: Ataluren	Phase 2	April 11, 2019	June 3, 2019
NCT02819557	Study of Ataluren in ≥ 2 to < 5 Year-Old Male Participants With Duchenne Muscular Dystrophy	Completed	Duchenne Muscular Dystrophy	Drug: Ataluren	Phase 2	June 9, 2016	February 9, 2018
NCT01247207	Study of Ataluren in Previously Treated Participants With Nonsense Mutation Dystrophinopathy (nmDBMD)	Enrolling by invitation	Duchenne Muscular Dystrophy	Drug: Ataluren	Phase 3	November 30, 2010	December 15, 2023
NCT00759876	Phase 2a Extension Study of Ataluren (PTC124) in	Terminated	Duchenne Muscular Dystrophy	Drug: Ataluren	Phase 2	August 13, 2008	May 17, 2010

	Duchenne Muscular Dystrophy (DMD)						
NCT00451074	Six Month Study of Gentamicin in Duchenne Muscular Dystrophy With Stop Codons	Completed	Duchenne Muscular Dystrophy	Drug: Gentamicin infusions twice a week for six months	Phase 1	March 2007	July 2009
NCT00264888	Safety and Efficacy Study of PTC124 in Duchenne Muscular Dystrophy	Completed	Duchenne Muscular Dystrophy	Drug: PTC124	Phase 2	December 2005	May 2007
NCT00847379	Phase 2B Extension Study of Ataluren (PTC124) in Duchenne/Becker Muscular Dystrophy (DMD/BMD)	Terminated	Duchenne Muscular Dystrophy	Drug: Ataluren	Phase 2	January 31, 2009	May 24, 2010
NCT01009294	Study of Ataluren (PTC124) in Nonambulatory Participants With Nonsense-Mutation-Mediated Duchenne/Becker Muscular Dystrophy (nmDMD/BMD)	Terminated	Duchenne Muscular Dystrophy	Drug: Ataluren Drug: Chronic Corticosteroid Therapy	Phase 2	January 13, 2010	March 23, 2010
NCT00592553	Phase 2B Study of PTC124 (Ataluren) in Duchenne/Becker Muscular Dystrophy (DMD/BMD)	Completed	Duchenne Muscular Dystrophy	Drug: Ataluren Drug: Placebo	Phase 2	February 29, 2008	December 31, 2009
NCT01557400	Study of Ataluren for Previously Treated Participants With Nonsense Mutation Duchenne/Becker Muscular Dystrophy	Completed	Duchenne Muscular Dystrophy	Drug: Ataluren	Phase 3	May 20, 2012	January 19, 2018

	(nmDBMD) in Europe, Israel, Australia, and Canada						
NCT01918384	Phase II Study of NPC-14 (Arbekacin Sulfate) to Explore Safety, Tolerability, and Efficacy in Duchenne Muscular Dystrophy	Unknown status	Duchenne Muscular Dystrophy	Drug: NPC-14 Drug: Placebo	Phase 2	August 1, 2013	October 2015
NCT03179631	Long-Term Outcomes of Ataluren in Duchenne Muscular Dystrophy	Active, not recruiting	Duchenne Muscular Dystrophy	Drug: Ataluren Drug: PLACEBO	Phase 3	July 6, 2017	July 20, 2023
NCT02090959	An Extension Study of Ataluren (PTC124) in Participants With Nonsense Mutation Dystrophinopathy	Terminated	Duchenne Muscular Dystrophy	Drug: Ataluren	Phase 3	March 20, 2014	June 12, 2018
NCT01826487	Phase 3 Study of Ataluren in Participants With Nonsense Mutation Duchenne Muscular Dystrophy (nmDMD)	Completed	Duchenne Muscular Dystrophy	Drug: Ataluren Drug: Placebo	Phase 3	March 26, 2013	August 20, 2015
NCT04336826	A Study to Evaluate the Safety and Pharmacokinetics of Ataluren in Participants From ≥ 6 Months to < 2 Years of Age With Nonsense Mutation Duchenne Muscular Dystrophy (nmDMD)	Recruiting	Duchenne Muscular Dystrophy	Drug: Ataluren	Phase 2	December 29, 2021	March 31, 2023
NCT04644627	Topical Gentamicin Nonsense Suppression Therapy of EB	Completed	Epidermolysis Bullosa	Drug: Gentamicin Sulfate	Phase 1 Phase 2	December 1, 2020	May 1, 2022

NCT02758626	Ataluren for Nonsense Mutation in CDKL5 and Dravet Syndrome	Completed	Epilepsy	Drug: ataluren Drug: Placebo	Phase 2	November 1, 2016	February 27, 2021
NCT04454151	Azithromycin Treatment for Readthrough of APC Gene Stop Codon Mutations in Familial Adenomatous Polyposis	Unknown status	Familial Adenomatous Polyposis	Drug: Azithromycin Tablets	Phase 4	August 1, 2020	April 1, 2022
NCT02175914	Erythromycin Treatment for Readthrough of APC Gene Stop Codon Mutations in Familial Adenomatous Polyposis	Unknown status	Familial Adenomatous Polyposis	Drug: Erythromycin	Phase 4	June 2014	June 2016
NCT02354560	Erythromycin Treatment for Readthrough of APC Gene Stop Codon Mutation in Familial Adenomatous Polyposis-minors' Adjusted Version	Unknown status	Familial Adenomatous Polyposis	Drug: Erythromycin	Phase 4	June 2015	December 2017
NCT03309605	Phase 1 Study of ELX-02 in Healthy Adult Subjects	Completed	Healthy	Drug: ELX-02 Drug: Placebo	Phase 1	October 11, 2017	July 17, 2019
NCT03292302	Phase 1 Study of ELX-02 in Healthy Adults	Completed	Healthy	Drug: Placebo Drug: ELX-02	Phase 1	September 26, 2017	December 15, 2017
NCT02807961	Safety, Tolerability, Pharmacokinetics of ELX-02 in Healthy Adult Volunteers	Terminated	Healthy	Drug: ELX-02 Drug: Placebo	Phase 1	July 2016	May 2017
NCT02409004	Effects of Rifampin on the Pharmacokinetics of Ataluren	Completed	Healthy	Drug: Ataluren Drug: Rifampin	Phase 1	February 2015	March 2015
NCT00947193	Study of Ataluren (PTC124) in Hemophilia A and B	Terminated	Hemophilia A Hemophilia B	Drug: Ataluren	Phase 2	October 14, 2009	August 30, 2011

NCT03492866	Efficacy of Topical Gentamycin for Hereditary Hypotrichosis Simplex Caused by Nonsense Mutations in CDSN	Unknown status	Hereditary Hypotrichosis Simplex	Drug: Gentamicin Sulfate	Phase 2	April 1, 2018	April 1, 2019
NCT03776539	A Study to Evaluate the Effects of Renal Impairment on the Pharmacokinetics of ELX-02	Completed	Impaired Renal Function	Drug: ELX-02	Phase 1	January 4, 2019	August 7, 2019
NCT03526159	Gentamicin for Junctional Epidermolysis Bullosa	Recruiting	Junctional Epidermolysis Bullosa	Drug: Gentamicin Sulfate	Phase 1 Phase 2	June 1, 2018	August 31, 2020
NCT04140786	Optimizing IV Gentamicin in JEB	Recruiting	Junctional Epidermolysis Bullosa	Drug: Gentamicin Sulfate, Injectable	Phase 1 Phase 2	October 31, 2019	November 1, 2023
NCT04069260	A Phase 2 Study of ELX-02 in Patients With Nephropathic Cystinosis	Terminated	Nephropathic Cystinosis	Drug: ELX-02	Phase 2	August 2, 2019	December 17, 2019
NCT03392909	Intravenous Gentamicin Therapy for Recessive Dystrophic Epidermolysis Bullosa (RDEB)	Recruiting	Recessive Dystrophic Epidermolysis Bullosa	Drug: Gentamicin	Phase 1 Phase 2	July 5, 2018	December 1, 2023
NCT03012191	Gentamicin for RDEB	Completed	Recessive Dystrophic Epidermolysis Bullosa	Drug: Gentamicin Sulfate	Phase 1 Phase 2	February 2, 2017	August 31, 2018
NCT02698735	Gentamicin Therapy for Recessive Dystrophic Epidermolysis Bullosa (RDEB) Nonsense Mutation Patients	Completed	Recessive Dystrophic Epidermolysis Bullosa	Drug: Gentamicin Drug: Placebo	Phase 1 Phase 2	February 25, 2016	June 30, 2017