Evaluating the Clinical Trial Option

A GUIDE TO CPN RESOURCES

Trial Basics		Topics include: Phases of a Trial; Roles of Sponsor, FDA, Principal Investigator, Study Coordinator; Method of Treatment/Delivery; Dose Escalation and Placebo Controlled Trials; Compassionate Use; Pioneers in Clinical Trials		
Format	Voice	Title	Link	Description
Video	Dawn Mother Canavan Disease	Clinical Trials: A mom on her child participating in a natural history study	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-on-her-child-partic ipating-in-a-natural-history-s tudy/	The mother of a 16-month-old with Canavan describes the process by which her child is participating in a natural history study.
Video	Diane McKenna-Ya sek Study Coordinator	Clinical Trials: Staggered enrollment, Cohorts and Dose Escalation	https://courageousparentsne twork.org/videos/clinical-trial s-staggered-enrollment-coh orts-and-dose-escalation/	A Study Coordinator explains that not all patients in a trial are treated at the same time; enrollment is staggered for a reason. She explains what the term cohort means and the process of dose escalation trials. She also talks about trials as a learning process.
Video	Diane McKenna-Ya sek Study Coordinator	Clinical Trials Sponsors, Internal Review Boards	https://courageousparentsne twork.org/videos/clinical-trial s-sponsors-internal-review-b oards/	A clinical trial Study Coordinator explains what a sponsor of a trial is and the role of the Internal Review Boards (IRB).

Format	Voice	Title	Link	Description
Video	Emily & Darren Parents Hurler Syndrome (MPS I)	Enzyme Replacement Therapy, Stem Cell Replacement and living into the unknown	https://courageousparentsne twork.org/videos/enzyme-re placement-therapy-stem-cell -transplant-and-living-into-th e-unknown	Parents of a 4-year old son with Hurler Syndrome talk about their decision to pursue a stem cell transplant. "For me it was, well Hurlers is a 100 percent fatal, stem cell transplants, whatever the mortality rate is, is lower which is better than a 100 percent mortality rate. We took a shot. The quality of life question is unanswerable so we didn't ask it."
Video	Jamie Ring Patient Advocate Bio-Tech	A Patient Advocate explains the Placebo controlled trial	https://courageousparentsne twork.org/videos/a-patient-a dvocate-explains-the-placeb o-controlled-trial/	A patient advocate in biotechnology discusses a placebo arm in a clinical trial. She talks about questions to ask when considering a trial that includes a placebo arm including if the trial includes a rescue clause.
Video	Jamie Ring Patient Advocate Bio-Tech	The role of a Patient Advocate at a biotech company	https://courageousparentsne twork.org/videos/the-role-of- a-patient-advocate-at-a-biot ech-company/	A patient advocate explains her role in the biotechnology industry.
Video	Jamie Ring Patient Advocate Bio-Tech	Everyone who participates in rare disease research is a medical hero	https://courageousparentsne twork.org/videos/everyone- who-participates-in-rare-dis ease-research-is-a-medical- hero/	A patient advocate in biotechnology talks about how the families who participate in research are pioneers and medical heroes. Patients' willingness to take the risk and contribute is the only way to forward research. "We (biotech) can build a rocket ship to take us to space, but we can't fly it. We need a volunteer."
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: A mom on dose escalation studies and gene therapy	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-on-dose-escalatio n-studies-and-gene-therapy/	The mother of two boys with Duchenne Muscular Dystrophy talks about putting a child in a dose escalating study. She explains the difficult decision of participating in a Phase 1 gene therapy trial at the lowest dose She briefly discusses placebo-controlled trials.

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Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: A mom: "Eventually we are going to be looking at combination therapies."	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-eventually-we-are- going-to-be-looking-at-com bination-therapies/	A mother of two sons with Duchenne Muscular Dystrophy talks about how eventually science will be looking at combination therapies – "adding to the next best treatment."
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: A mom: We can't ignore the economics	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-we-cant-ignore-th e-economics/	A mother of two sons with Duchenne Muscular Dystrophy and a son with a Primary Immune Deficiency talks about why we cannot ignore the economics of drug development and how individuals advocating to insurance companies can help ensure that they have accurate knowledge about your disease.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: The logistics and limitations of gene therapy	https://courageousparentsne twork.org/videos/clinical-trial s-the-logistics-and-limitation s-of-gene-therapy/	A mother of a child with Sanfilippo Syndrome talks about gene therapy as a "one and done" treatment and acknowledges the limitations of developing the antibodies to the vector eliminating re-dosing as an option. With two children being screened for gene therapy, she talks about the logistics of keeping the children separated once the first child had been treated.
Video	Michelle & Joe Parents Battens Disease (CLN2)	Clinical Trials: How enzyme replacement therapy was delivered	https://courageousparentsne twork.org/videos/cln2-batten -how-the-ert-is-delivered	Parents of two sons, one of whom has CLN2 (Batten disease), describe how his port goes into the ventricles in his brain and how the infusions are delivered.

Format	Voice	Title	Link	Description
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: Defining Compassionate Use, Expanded Access	https://courageousparentsne twork.org/videos/clinical-trial s-defining-compassionate-u se-expanded-access/	A Principal Investigator explains pathways outside of the standard clinical trial pathways; expansion studies, expanded access and compassionate use. He stresses that most often these are not considered until a Phase I trial is complete and safety is known. Compassionate Use is not a substitute for the clinical trial pathway, however he acknowledges circumstances when it might be employed.
Video	Dr. Terry Flotte Principal Investigator	Compassionate use is no substitute for the trial pathway	https://courageousparentsne twork.org/videos/compassio nate-use-is-no-substitute-for -the-trial-pathway/	A Principal Investigator discusses Compassionate Use. He explains that it should not be a substitute for the trial pathway which often includes more opportunities for scientific learning.
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials are designed to get to FDA approval ASAP	https://courageousparentsne twork.org/videos/clinical-trial s-are-designed-to-get-to-fda -approval-asap/	A Principal Investigator explains that because trials in rare disease are designed to get to FDA approval as quickly as possible, the initial number and criteria for patients is likely to be narrow to increase chances of success which will benefit the greater good.
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: Dose escalation, Phase by Phase	https://courageousparentsne twork.org/videos/clinical-trial s-dose-escalation-phase-by -phase/	A Principal Investigator explains a dose escalating trial; the purpose is to determine dose related side effects and assure safety at each phase while at the same time providing a reasonable benefit to the participant. He acknowledges that families sometimes struggle with the decision to participate in early phases.

Format	Voice	Title	Link	Description
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: Phase 1 defined – focusing on safety, with less certainty about benefit. Phase 1 trials for rare disease are designed for some efficacy	https://courageousparentsne twork.org/videos/clinical-trial s-phase-1-defined-focusing- on-safety-with-less-certainty -about-benefit-phase-1-trials -for-rare-disease-are-design ed-for-some-efficacy/	A Principal Investigator discusses the different phases of a trial. He explains that early phase trials are focused on safety as the primary endpoint where later phases trials have a greater focus on efficacy. He uses the terms "first in human", "dose finding" and "risk/benefit balance". He explains that with many trials for rare diseases, the phases may be combined.
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: With Gene Therapy, it is ONE and DONE	https://courageousparentsne twork.org/videos/clinical-trial s-with-gene-therapy-it-is-on e-and-done/	A Principal Investigator talks about gene therapy, the immune response, and explains that with the current state technology, if you have been dosed once (even at a low dose) you usually cannot be dosed again. He acknowledges that this is often a factor in the decision to participate in a trial.
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: In the early phase, it's about both gaining knowledge and trying to help. To figure it out, we need some families to step up and go first	https://courageousparentsne twork.org/videos/clinical-trial s-in-the-early-phase-its-abo ut-both-gaining-knowledge- and-trying-to-help-to-figure-i t-out-we-need-some-familie s-to-step-up-and-go-first/	A Principal Investigator explains that clinical trials are about both research and therapy; their goal is to gain knowledge and help the participants. Trials are a process and at each step, researchers learn things. He stresses that research needs families to step up and agree to be in the early phases; those families are trailblazers. He acknowledges that when trial outcomes are not met, participants often feel like the trial is a failure but emphasizes that at each step scientific knowledge is gained.
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: The Role of the Principal Investigator, Sponsor, IRB and FDA	https://courageousparentsne twork.org/videos/clinical-trial s-the-role-of-the-principal-in	A Principal Investigator provides a brief description of the various roles involved in a trial.

		vestigator-sponsor-irb-and-f da/		
Design	Topics Include: Trial Protocols; Endpoints; Inclusion/Exclusion Criteria; Efficacy			
Dawn Mother Canavan Disease	A Canavan mom on learning gene therapy was on the horizon and getting information from the company. "Information is being shared the way it needs to be shared."	https://courageousparentsne twork.org/videos/a-canavan- mom-on-learning-gene-thera py-was-on-the-horizon-and- getting-information-from-the -company-information-is-bei ng-shared-the-way-it-needs- to-be-shared/	The mother of a 16-month old talks with Courageous Parents Network about how she first learned of potential gene therapy for her daughter and how the Sponsor company is keeping the Canavan community informed.	
Jamie Ring Patient Advocate Bio-Tech	Balancing hope and realistic expectations in clinical trials	https://courageousparentsne twork.org/videos/balancing- hope-and-realistic-expectati ons-in-clinical-trials/	A patient advocate in biotechnology discusses how patient advocates can help families feel excited and hopeful about clinical research while at the same time balancing realistic expectations. Clinical research is a process, and even if the trial is considered unsuccessful or the outcome is not what was hoped for, something is always learned.	
Jamie Ring Patient Advocate Bio-Tech	Community input is valued but every trial design is unique	https://courageousparentsne twork.org/videos/community -input-is-valued-but-every-tri al-design-is-unique/	A patient advocate in biotechnology discusses the valued input of the patient community in clinical trials, explaining that every trial design is unique and the process varies company to company, phase to phase, and between different types of investigational products. She also touches on the formality of many of the steps required in gene therapy trials.	
Jamie Ring Patient Advocate	Endpoints and the patient perspective in designing trials	https://courageousparentsne twork.org/videos/endpoints-	A patient advocate in biotech talks about the importance of the patient perspective in	
	Dawn Mother Canavan Disease Jamie Ring Patient Advocate Bio-Tech Jamie Ring Patient Advocate Bio-Tech	DesignTrial Protocols; EndpoinDawn Mother Canavan DiseaseA Canavan mom on learning gene therapy was on the horizon and getting information from the company. "Information is being shared the way it needs to be shared."Jamie Ring Patient Advocate Bio-TechBalancing hope and realistic expectations in clinical trialsJamie Ring Patient Advocate Bio-TechCommunity input is valued but every trial design is uniqueJamie Ring Patient Advocate Bio-TechCommunity input is valued but every trial design is uniqueJamie Ring Patient Advocate Bio-TechEndpoints and the patient perspective in	da/DesignTopics Include: Trial Protocols; Endpoints; Inclusion/Exclusion Criteria;Dawn Mother Canavan DiseaseA Canavan mom on learning gene therapy was on the horizon and getting information from the company. "Information is being shared the way it needs to be shared."https://courageousparentsne twork.org/videos/a-canavan- mom-on-learning-gene-thera py-was-on-the-horizon-and- getting-information-from-the -company-information-is-bei ng-shared-the-way-it-needs- to-be-shared/Jamie Ring Patient Advocate Bio-TechBalancing hope and realistic expectations in clinical trialshttps://courageousparentsne twork.org/videos/balancing- hope-and-realistic-expectati ons-in-clinical-trials/Jamie Ring Patient Advocate Bio-TechCommunity input is valued but every trial design is uniquehttps://courageousparentsne twork.org/videos/community -input-is-valued-but-every-tri al-design-is-unique/Jamie Ring Patient Advocate Bio-TechEndpoints and the patient perspective inhttps://courageousparentsne twork.org/videos/community	

	Bio-Tech		and-the-patient-perspective- in-designing-trials/	determining outcomes and endpoints for clinical trials.
Format	Voice	Title	Link	Description
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: Inclusion and Exclusion criteria defined	https://courageousparentsne twork.org/videos/clinical-trial s-inclusion-and-exclusion-cr iteria-defined-2/	A Principal Investigator explains inclusion criteria and exclusion criteria. He explains who determines the criteria, defining both the role of the investigator and the sponsor. He acknowledges that sometimes individuals/families feel frustration with criteria and left out when they do not qualify for a trial.
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: Efficacy and End Points defined. End points are indicators on the road to efficacy	https://courageousparentsne twork.org/videos/clinical-trial s-efficacy-and-end-points-d efined-end-points-are-indica tors-on-the-road-to-efficacy/	A Principal Investigator explains the FDA's definition of efficacy. He talks about the importance of endpoints in clinical trials as indicators on the road to efficacy. He describes the range of things that could be considered when determining endpoints.
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: Limited slots - first come, first served and screened re criteria	https://courageousparentsne twork.org/videos/clinical-trial s-limited-slots-first-come-fir st-served-and-screened-re-c riteria/	A Principal Investigator explains why clinical trials, particularly gene therapy trials, often include a limited number of participants and that often in screening it is a "first come, first served" process when there are several participants who meet the inclusion criteria. He also discusses vector production.
Video	Dawn Mother Canavan Disease	Clinical Trials: A mom – "I hope I get to make that decision whether to participate and that I make the right decision."	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-i-hope-i-get-to-ma ke-that-decision-whether-to- participate-and-that-i-make- the-right-decision/	The mother of a 16-month old articulates the challenge that parents of children considering early stage clinical trials face: what is the right decision for my child in the absence of a crystal ball?

Qualifying for a Trial		Topics Include: Informed Consent; Screening process: Meeting the Eligibility Requirements; Exclusion from a Trial; Assent to Participate; Isolation Due to Participation; Prioritizing Activities to Meet Criteria			
Format	Voice	Title	Link	Description	
Video	Diane McKenna-Ya sek Study Coordinator	Clinical Trials: Recruitment and Pre-Screening	https://courageousparentsne twork.org/videos/clinical-trial s-recruitment-and-pre-scree ning/	A Study Coordinator explains the process of recruiting participants for a clinical trial; having an informal pre-screening or informational call with a Study Coordinator to understand the inclusion criteria, commitments necessary for participation, and gather the medical history of the potential participant.	
Video	Diane McKenna-Ya sek Study Coordinator	The Screening Visit: You Will Be Asked to Sign the Informed Consent Form, the most important document	https://courageousparentsne twork.org/videos/the-screeni ng-visit-you-will-be-asked-to -sign-the-informed-consent- form-the-most-important-do cument/	A Study Coordinator explains the screening visit; the visit where they determine if a potential participant meets the inclusion criteria for a clinical trial. She explains the purpose of the Informed Consent and provides helpful tips about understanding the document and preparing for the meeting where you will be asked to sign it.	
Video	Jamie Ring Patient Advocate Bio-Tech	Understanding the Why Behind Inclusion and Exclusion Criteria	https://courageousparentsne twork.org/videos/understand ing-the-why-behind-inclusio n-and-exclusion-criteria/	A patient advocate in biotechnology discusses inclusion criteria and why finding appropriate candidates for a trial is important to a safe, successful trial and how it often results in a more rapid acceleration of approved treatments. She talks about the heartbreaking reality that in choosing candidates the focus has to be on the greater good of the entire community over the individual.	

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Video	Jamie Ring Patient Advocate Bio-Tech	Informed consent. Take it seriously.	https://courageousparentsne twork.org/videos/informed-c onsent-take-it-seriously/	A patient advocate in biotechnology discusses Informed Consent. She advises patients/parents to take it seriously, take their time in reading it, consider sharing it with people who can help to understand it, and not to feel pressured or rushed to sign it.
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: Mom finds out about a trial on Facebook, learns about the trial design & the screening process	https://courageousparentsne twork.org/videos/clinical-trial s-mom-finds-out-about-a-tri al-on-facebook-learns-about -the-trial-design-the-screeni ng-process/	A mother of two sons with Duchenne Muscular Dystrophy talks about finding out about a trial on Facebook, screening, and trial design. She speaks about why it is important to enroll the right candidates in trial. She also discusses the misconception that you can "train" your child to meet the inclusion criteria, in this case a walk test.
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: A mom: Don't feel rushed, don't feel desperate – read the Informed Consent	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-dont-feel-rushed-d ont-feel-desperate-read-the- informed-consent/	A mother of two sons with Duchenne Muscular Dystrophy talks about the consent process and how parents/patients often ignore it because they feel rushed and desperate. She talks about what is included in the Informed Consent, the importance of asking questions, taking your time, and understanding what you are signing up for.
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: Assent to Participate: My son is old enough to decide for himself	https://courageousparentsne twork.org/videos/clinical-trial s-assent-to-participate-my-s on-is-old-enough-to-decide- for-himself/	A mother of two sons with Duchenne Muscular Dystrophy talks about the Assent to Participate, a document that children over a certain age with developmental capability must sign to receive a treatment or procedure. She also talks about being honest with her boys and asking them questions about procedures and trials. She

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Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: Excitement to participate can blind you to the details	https://courageousparentsne twork.org/videos/clinical-trial s-excitement-to-participate- can-blind-you-to-the-details/	A mother of two sons with Duchenne Muscular Dystrophy talks about the excitement and pressure families feel when there is the possibility of participating in a clinical trial and how that excitement can blind you to the important things like attending to the Informed Consent.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: Restrictions in the Informed Consent and participation can be isolating	https://courageousparentsne twork.org/videos/clinical-trial s-restrictions-in-the-informe d-consent-and-participation- can-be-isolating/	A mother of two children with Sanfilippo Syndrome discusses how participating in a clinical trial can be isolating. Not only do many trials contain restrictions about discussing your participation publicly but also because of some of the lifestyle changes that are necessary when participating.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: Prioritizing Activities to meet the eligibility criteria	https://courageousparentsne twork.org/videos/clinical-trial s-prioritizing-therapies-to-m eet-the-eligibility-criteria/	A mother of two children with Sanfilippo Syndrome talks about prioritizing her children's physical and speech therapies in the hopes that it would increase their performance during the screening process so they would meet the eligibility criteria. She talks about the importance of accurately representing your child so as not to risk jeopardizing the trial.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: Two children screened, one qualifies – antibodies exclude the other.	https://courageousparentsne twork.org/videos/clinical-trial s-two-children-screened-on e-qualifies-antibodies-exclu de-the-other/	A mother of two children with Sanfilippo Syndrome discusses the screening process, explains that her younger child, a son, met the inclusion criteria but then screened positive for antibodies to the vector and was excluded. She discusses isolating her other child so as not to risk her exposure to the virus and developing the antibodies during her screening.

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Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: Informed Consent defined. Parents and providers together making the best decision based on the limited information available	https://courageousparentsne twork.org/videos/clinical-trial s-informed-consent-defined- parents-and-providers-toget her-making-the-best-decisio n-based-on-the-limited-infor mation-available/	A Principal Investigator talks about the Informed Consent process as one where patients or parents have conversations about the risks and benefits of the treatment proposed in the clinical trial with the goal of making the best decision for the patient/family. It is about giving informed choices to families, explaining the unknown aspects of a trial, and asking the family to exercise their autonomy in making a decision.
Decision Making		Topics Include: Discussions on Willingne Family, Only One Qualifi		Right Trial at Right Time; Two Children, One
Podcast	Dawn Mother Canavan Disease	Dawn and Blyth: Anticipating a Clinical Trial	https://courageousparentsne twork.org/podcasts/podcast -dawn-and-blyth-anticipatin g-a-clinical-trial	Two mothers, one with a living child with Canavan and another with a deceased daughter with Tay-Sachs discuss progress in science, the complex decision to participate, creating a good team of experts to advise parents including industry partners and the hope that you will make the right decision.
Video	Emily & Darren Parents Hurler Syndrome (MPS I)	As a parent you do what you have to do	https://courageousparentsne twork.org/videos/as-a-paren t-you-do-what-you-have-to- do	Parents of a 4-year old son with Hurler Syndrome who has received a stem cell transplant talk about staying present and getting through his transplant.

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Video	Dawn Mother Canavan Disease	Clinical Trials: A mom considers her risk aversion. "For me it's more about whether this is the right thing for her."	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-considers-her-risk -aversion-for-me-its-more-a bout-whether-this-is-the-righ t-thing-for-her/	The mother of a 16-month old shares how she is thinking about what she hopes will be the upcoming opportunity for her daughter to participate in a gene therapy trial and what will determine her decision to participate.
Video	Diane McKenna-Ya sek Study Coordinator	Clinical Trials: Weighing the Risks and Benefits	https://courageousparentsne twork.org/videos/clinical-trial s-weighing-the-risks-and-be nefits/	A Study Coordinator on weighing the risks and benefits of participating in a clinical trial; includes evaluating how you will feel about any discomforts that may be a result of the trial procedures, the changing relationship within your disease community if you participate, and how you might feel about the unknown of the outcomes of a trial.
Blog	Glenn O'Neill Father Sanfilippo Syndrome (MPS III)	A Terminal Diagnosis and Risk Aversion	https://courageousparentsne twork.org/blog/a-terminal-di agnosis-and-risk-aversion	In this blog a father discusses his daughter participating in a Phase I, dose escalating gene therapy trial, and describes how they evaluated their risk aversion and made the decision to participate in the trial.
Video	Greg & Heidi Parents Leigh's Disease	Finding an Experimental Trial, Study Drug	https://courageousparentsne twork.org/videos/finding-an- experimental-trial-study-dru g-2	The mother and father of a 5-year old with Leigh's Disease, a mitochondrial condition, talk about how they did loads of research online, on their own, including Google Alerts, and learned about an experimental trial. "We had to educate ourselves in order to provide the best for our child."

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Video	Jamie Ring Patient Advocate Bio-Tech	Should I participate in a clinical trial?	https://courageousparentsne twork.org/videos/should-i-pa rticipate-in-a-clinical-trial/	A patient advocate in biotechnology discusses common concerns surrounding participation in a clinical trial with the goal of answering the underlying question: "How do I decide if I should participate?" Understanding what the research is trying to accomplish and discussing it with a physician, experts, industry sponsors and patient advocates can help patients make the decision.
Video	Jamie Ring Patient Advocate Bio-Tech	The Personal aspects of a clinical trial, including relocation, impact on other children	https://courageousparentsne twork.org/videos/the-person al-aspects-of-a-clinical-trial-i ncluding-relocation-and-the- impact-on-other-children/	A patient advocate in biotechnology discusses the personal aspects of clinical trial participation including relocation, impact on other children, and marriage. She advises making decisions as they come and not get too far ahead in the process.
Video	Jamie Ring Patient Advocate Bio-Tech	Trial Participation, balancing risk and reward, hope and expectation, commitment and community	https://courageousparentsne twork.org/videos/trial-partici pation-balancing-risk-and-re ward-hope-and-expectation- commitment-and-communit y/	A patient advocate in biotechnology discusses the very personal decision to participate in clinical trials and stresses that families should take their time to think about the long-term commitments and understand the balance of risks and rewards, hopes and expectations. She acknowledges that parents often feel burdened by the decision.
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: Deciding if this is the right trial – Are you looking for curative or something that mitigates? What if there is a placebo?	https://courageousparentsne twork.org/videos/clinical-trial s-deciding-if-this-is-the-right -trial-are-you-looking-for-cur ative-or-something-that-miti gates-what-if-there-is-a-plac ebo/	A mother of two sons with DMD offers guidance on deciding to participate. She asks, "are you looking for- curative treatments vrs mitigating symptoms?"; "What can your children tolerate the tests required?"; "Can your family/marriage survive the lifestyle changes you may need to make?"; "What if it is a trial with a placebo arm – can you handle your child getting the placebo?"

				She encourages asking questions of the trial sponsor, doctors, and the disease community.
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Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: A mom on weighing risk, understanding endpoints and managing expectations	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-on-weighing-risk-u nderstanding-endpoints-and -managing-expectations/	A mother of two sons with Duchenne Muscular Dystrophy discusses the fact that all trials involve some measure of risk. She used her understanding of the trial's endpoints to evaluate her risk tolerance and set her expectations about outcomes. "This is being studied because we don't know if it works." She also discusses risk in relation to the phase of the trial and safety trials.
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: A mom on assessing risk when her child has a treatable disease	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-on-assessing-risk- when-her-child-has-a-treata ble-disease/	A mother of two sons with Duchenne Muscular Dystrophy and a son with a Primary Immune Deficiency, talks about evaluating her risk tolerance in finding a curative treatment for her son with PID. She talks about the barriers and biases when your child's disease is considered a chronic, manageable condition and the limitations on the quality of her son's life.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: As she aged my expectations about what treatment would mean adjusted	https://courageousparentsne twork.org/videos/clinical-trial s-as-she-aged-my-expectati ons-about-what-treatment-w ould-mean-adjusted/	The mother of two children with Sanfilippo Syndrome talks about her pre-trial expectations for the outcomes of a treatment, how they adjusted as her daughter progressed in her disorder. She wonders at what point she would have chosen not to participate in an intervention.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: What diseases can actually say there is a cure?	https://courageousparentsne twork.org/videos/clinical-trial s-what-diseases-can-actuall y-say-there-is-a-cure/	The mother of two children with Sanfilippo Syndrome discusses using the word cure in an aspirational way while fundraising, but is careful using it when talking about treatments or therapies – What diseases can actually say there is a cure?

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Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: Which child? The decision was made for me.	https://courageousparentsne twork.org/videos/clinical-trial s-which-child-the-decision- was-made-for-me/	A mother discusses having two children with a metabolic disorder, Sanfilippo Syndrome. Her youngest screened for a gene therapy trial but it was found he possessed the antibodies to the vector and could not participate. Her older daughter was able to participate. She never had to choose a child because the antibodies made the decision for her.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: Whatever decision you make, it is going to be right for you	https://courageousparentsne twork.org/videos/clinical-trial s-whatever-decision-you-ma ke-it-is-going-to-be-right-for -you/	The mother of two children with Sanfilippo Syndrome talks about how advancements in science have made it so that some families will be faced with choosing which trial to participate in. She discusses pursuing all the available options but emphasizes feeling confident that when the time comes to choose, you will make the decision that is right for you.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: Is this really the right thing for my child?	https://courageousparentsne twork.org/videos/clinical-trial s-is-this-really-the-right-thin g-for-my-child/	The mother of two children with Sanfilippo Syndrome talks about understanding the risks during the Informed Consent process. She speaks honestly about the pressure that "something is better than nothing" and about deciding if this was really the right thing for her child. She remembers thinking "if we could just keep her like this her life would be ok."
Video	Michelle & Joe Parents Battens Disease	Clinical Trials: Weighing the Risks; A Leap of Faith	https://courageousparentsne twork.org/videos/clinical-trial s-weighing-the-risks-a-leap- of-faith-cln2-batten	Parents of two sons, one of whom has CLN2 (Batten disease), talk about how they considered enrolling their young son in the trial: they learned from a previous trial in Europe. "When you get a diagnosis that your child is going to pass away,

	(CLN2)			the side effects are not a top priority. It goes back to doing something. Let's do this."
Format	Voice	Title	Link	Description
Video	Steve and Sarah Parents Gaucher Type II	Now I can have the long view towards finding a treatment or cure	https://courageousparentsne twork.org/videos/now-i-can- have-the-long-view-towards -finding-a-treatment-or-cure /	The mother of a daughter with Gaucher Type II reflects on how she focused on quality of life during her daughter's short time. But now that her daughter has died, she can look out towards the long road to a cure.
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: Timing is everything. Participate early or wait for Phase 3?	https://courageousparentsne twork.org/videos/clinical-trial s-timing-is-everything-partici pate-early-or-wait-for-phase -3/	A Principal Investigator talks about the difficult decision of determining if this is the right trial at the right time for your child, particularly if they have a progressive rare disease and may not meet the inclusion criteria when later phases of a trial begin enrolling.
Emotions and Questions				on Disease Community; Advocating for your Child; and Siblings; Natural History Study
Blog	Daniel Father Menkes Disease	The Cure We Don't Want	https://courageousparentsne twork.org/blog/author/daniel -defabio	In this blog, a father acknowledges that his son may be too far progressed in his disorder to participate in a clinical trial and that even if he were chosen to participate, he might decline it.
Video	Dawn Mother Canavan Disease	A Rare Disease mom: Just tell me, what do I need to do?	https://courageousparentsne twork.org/videos/clinical-trial s-a-rare-disease-mom-just-t ell-me-what-do-i-need-to-do /	The mother of a 16-month old with Canavan disease expresses the sentiment that all parents of children with rare disease feel: There must be something I can do. She discusses fundraising,

		and advocating with the FDA. She acknowledges that much of the process is out of her control.

Format	Voice	Title	Link	Description
Podcast	Diana Pangonis Director of Family Services, NTSAD	A Discussion with Diana Pangonis, NTSAD	https://courageousparentsne twork.org/podcasts/podcast -jennifer-and-diane-supporti ng-families-through-clinical-t rial	CPN's Outreach Manager Jennifer Siedman and Diana Pangonis, NTSAD's Director of Family Services, have a conversation about the new landscape of clinical trial research for the NTSAD community, the common issues that often arise as a result in disease communities and helping families navigate the uncharted territory.
Video	Diane McKenna-Ya sek Study Coordinator	Study Coordinator Talks About Commonly Asked Questions - Site Visits, and Access	https://courageousparentsne twork.org/videos/study-coor dinator-talks-about-common ly-asked-questions-site-visit s-and-access/	A Study Coordinator addresses some commonly asked questions including questions about study visits, open label access, and financial considerations of participating.
Video	Diane McKenna-Ya sek Study Coordinator	Clinical Trials: a Study Coordinator reflects on the Emotional Costs	https://courageousparentsne twork.org/videos/clinical-trial s-a-study-coordinator-reflect s-on-the-emotional-costs/	A Study Coordinator reflects on the emotional cost of trial participation and stresses the importance of helping participants find support during trials.
Video	Diane McKenna-Ya sek Study Coordinator	Clinical Trials: The Role of Natural History Studies	https://courageousparentsne twork.org/videos/clinical-trial s-the-role-of-natural-history- studies/	A Study Coordinator discusses the important role Natural History Studies play in advancing the research that leads to clinical trials and stresses the importance of participating in them.
Video	Jamie Ring Patient Advocate Bio-Tech	Managing Expectations: Trials are a learning process and timelines change	https://courageousparentsne twork.org/videos/managing- expectations-trials-are-a-lea rning-process-and-timelines -change/	A patient advocate in biotechnology discusses the importance of managing expectations especially around timelines and deadlines in clinical research. "On the road map of drug development, particularly in rare disease, there are a lot of detours and while we hope things will

				proceed as planned, trials are a learning process. Patients can sometimes feel disappointed in the timelines so there is no point in having deadlines."
Format	Voice	Title	Link	Description
Video	Jamie Ring Patient Advocate Bio-Tech	A strong, unified disease community is key	https://courageousparentsne twork.org/videos/clinical-trial s-a-strong-unified-disease-c ommunity-helps-stay-active- and-engaged-even-if-your-s ubgroup-doesnt-yet-have-a- therapy/	A patient advocate in biotechnology discusses why a strong, unified disease community is key. Staying active and engaged is important, even if your sub-group of the disease community does not yet have a treatment.
Video	Jamie Ring Patient Advocate Bio-Tech	The hard truth is that not everyone will qualify or meet the inclusion criteria	https://courageousparentsne twork.org/videos/the-hard-tr uth-is-that-not-everyone-will -qualify-or-meet-the-inclusio n-criteria/	A patient advocate in biotechnology acknowledges how hard it can be for families to accept that their child may not get into a trial and explains that it's not personal to the individual child or family.
Video	Jamie Ring Patient Advocate Bio-Tech	For the patient that doesn't qualify, there are other ways to participate	https://courageousparentsne twork.org/videos/for-the-pati ent-that-doesnt-qualify-there -are-other-ways-to-participa te/	A patient advocate in biotechnology discusses the heartbreaking conversations with families who do not qualify for a trial and stresses the importance of finding other ways to be involved in the community and participate in the advancement of research.
Video	Jamie Ring Patient Advocate Bio-Tech	How can I best advocate for my child?	https://courageousparentsne twork.org/videos/how-can-i- best-advocate-for-my-child/	A patient advocate in biotechnology discusses the most productive ways to advocate for your child in the clinical research process.
Video	Jenn Mother	Clinical Trials: Child Life can help make the trial experience better for your child	https://courageousparentsne twork.org/videos/clinical-trial s-child-life-can-help-make-t	A mother of two sons with Duchenne Muscular Dystrophy describes how she made participating in a clinical trial more tolerable for her son by utilizing Child Life Specialists.

		Duchenne Muscular Dystrophy		<u>he-trial-experience-better-fo</u> <u>r-your-child/</u>	
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Format	Voice	Title	Link	Description
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: My son participated in double-blind placebo studyI did not anticipate the extended trial timeline	https://courageousparentsne twork.org/videos/clinical-trial s-my-son-participated-in-do uble-blind-placebo-studyi-di d-not-anticipate-the-extende d-trial-timeline/	A mother of two sons with Duchenne Muscular Dystrophy talks about only one of her sons meeting the inclusion criteria and qualifying for the study as well as her naivety about how often timelines in studies change. She explains a double-blind placebo study and how in her son's study, all the participants began receiving the drug once evidence proved safety and efficacy. She talks about the FDA process and the importance of knowing the sponsors plan for the period of time between study completion and approval of the treatment.
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: A mom: Sometimes if feels like there are "Haves" and "Have Nots"	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-sometimes-if-feels -like-there-are-haves-and-ha ve-nots/	A mother of two sons with Duchenne Muscular Dystrophy talks honestly about how often in rare disease it feels like there are "haves" and "have-nots" in the community.
Podcast	Leslie Urdaneta Family Program Coordinator National MPS Society	Interview with Leslie Urandeta, MPS Society	https://courageousparentsne twork.org/podcasts/jennifer- and-leslie-community-and-cl inical-trials	. In this episode, CPN's Jennifer Siedman talks with Leslie Urdanta, Family Support Coordinator for the National MPS Society, a community for individuals and families living with MPS and ML about how the Society supports its families through the relatively new landscape of clinical trials; managing expectations, tempering frustrations, lifting up its members during times of disappointment, celebrating with them when advancements happen and always keeping the community united.

Format	Voice	Title	Link	Description
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: Waiting for an emerging therapy	https://courageousparentsne twork.org/videos/clinical-trial s-waiting-for-an-emerging-th erapy/	A mother of two children with Sanfilippo Syndrome discusses waiting for emerging enzyme replacement and gene therapy research to come to a trial. She discusses how the timelines in clinical research are long, much longer than she expected, and how stressful it was not knowing if a trial would come in time for her children to participate.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: Survivor's guilt and my place in the community.	https://courageousparentsne twork.org/videos/clinical-trial s-survivors-guilt-and-my-pla ce-in-the-community/	A mother discusses survivors' guilt and the perception that members of the disease community might see her child as having qualified because of her role in funding research.
Blog	Melissa Hogan Hunters Syndrome (MPS II)	Survivor's Guilt	https://courageousparentsne twork.org/blog/what-survivor s-guilt-looks-like-in-rare-dise ase	The mother of a son with Hunters Syndrome shares her feelings of survivor guilt after her son was included in an enzyme replacement trial.
Video	Michelle & Joe Parents Battens Disease (CLN2)	Clinical Trials: You adjust your life and you just do it	https://courageousparentsne twork.org/videos/clinical-trial s-you-adjust-your-life-and-ju st-do-it-cln2-batten	Parents of two sons, one of whom has CLN2, describe the ERT protocol during the first year living away from home for the trial. Mom notes that you never know how long the trial is going to take.
Video	Michelle & Joe Parents	Clinical Trials: We really felt like we had the golden ticket	https://courageousparentsne twork.org/videos/clinical-trial s-we-really-felt-like-we-had-	Parents of two sons, one of whom has CLN2, talk about some of the emotions they felt knowing that their son was in the trial while other children they had come to know and care for were not. They

	Battens Disease (CLN2)		<u>the-golden-ticket-cln2-batte</u> <u>n</u>	discuss the FDA process, the business and costs of the research industry.
Format	Voice	Title	Link	Description
Video	Michelle & Joe Parents Battens Disease (CLN2)	Clinical Trials: Relocation and impact on sibling	https://courageousparentsne twork.org/videos/clinical-trial s-relocation-and-impact-on- the-sibling-batten-cln2	Parents of two sons, one of whom has CLN2, talk about how they managed the year apart - when they were separated during the trial - for their older son who remained at home with dad; what they explained to him, how they supported him, how they managed to meet up periodically.
Video	Michelle & Joe Parents Battens Disease (CLN2)	Clinical Trials: Holding onto Hope (compilation video)	https://courageousparentsne twork.org/videos/cln2-batten -clinical-trials-expectations- holding-on-to-hope	Parents of two sons, one of whom has CLN2 and is receiving enzyme replacement therapy, share how they think about the future. Mom describes the shift in the disease community now that there is an approved treatment and gene therapy trials on the horizon.
Video	Dr. Terry Flotte Principal Investigator	Clinical Trials: Inclusion and Exclusion Criteria; Age and Ability of the child are often key factors	https://courageousparentsne twork.org/videos/clinical-trial s-inclusion-and-exclusion-cr iteria-age-and-ability-of-the- child-are-often-key-factors/	A Principal Investigator talks about inclusion criteria where age and ability are often key factors. He discusses the difficult quandary - it is hard for parents to hear (and for researchers to deliver) that their child's disease progression might be too great to participate in a trial or that the treatment might not offer enough clinical benefit.
After Trial		Topics Include: Living into an unknown	prognosis; Leaving Legacy	
Video	Michelle & Joe Parents	Clinical Trials: You still need to be an advocate for your child	https://courageousparentsne twork.org/videos/cln2-batten -clinical-trials-you-still-need-	Parents of two sons, one of whom has CLN2, offer advice to parents about participating in a trial - know the timeline, advocate for your child and your family. They need us, the child in the

Battens to-be-an-advocate-for-your- trial, as much as we need them, the or Disease child delivering the treatment. "We get to see the sector of the treatment."	
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Format	Voice	Title	Link	Description
Video	Emily & Darren Parents Hurler Syndrome (MPS I)	Post Transplant: We don't know what the future brings and how we will handle it	https://courageousparentsne twork.org/videos/we-dont-k now-what-the-future-holds-a nd-how-we-handle-that-post -stem-cell-transplant	Parents of a 4-year old son with Hurler Syndrome, who has received a stem cell transplant, talk about how they handle living into an uncertain future for their son. "We know he's going to need a lot of surgeries in the future. What's next? We don't have that crystal ball and that drives me nuts." "We don't ask these questions about our healthy son so I try not to think about it with our son who has a diagnosis."
Video	Greg & Heidi Parents Leigh's Disease	When Your Child Is in an experimental trial	https://courageousparentsne twork.org/videos/when-your- child-is-in-an-experimental-c linical-trial-2	The parents of a 5-year old with Leigh's Disease talk about how their daughter's positive response to an experimental study drug means she has more time, but they don't know how much or what to expect.
Video	Jenn Mother Duchenne Muscular Dystrophy	Clinical Trials: A mom: I didn't worry about disease progression then and I don't post trial	https://courageousparentsne twork.org/videos/clinical-trial s-a-mom-i-didnt-worry-abou t-disease-progression-then- and-i-dont-post-trial/	A mother of two sons with Duchenne Muscular Dystrophy, both of whom now receive drug treatments, explains how she does not really worry about the disease progression; how there were no guarantees in expected timelines pre-treatment and there are none post. She also talks about her son's peers dying and the guilt associated with participating in a trial.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: You give up some control when your child is a data point	https://courageousparentsne twork.org/videos/clinical-trial s-you-give-up-some-control -when-your-child-is-a-data- point/	A mother talks about how when your child is in a clinical trial much of the information or data collected is not shared with the parents and how difficult it is to look at the reports the trial sponsor posts and wonder which data point represents your child.

Format	Voice	Title	Link	Description
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: Once treated the road ahead is unknown	https://courageousparentsne twork.org/videos/clinical-trial s-once-treated-the-road-ahe ad-is-unknown/	A mother of two children with Sanfilippo Syndrome talks about how once her daughter was treated in a clinical trial the road ahead and her daughter's disease progression was not known.
Video	Megan Mother Sanfilippo Syndrome (MPS III)	Clinical Trials: The pioneers and those who come after	https://courageousparentsne twork.org/videos/clinical-trial s-the-pioneers-and-those-w ho-come-after/	A discussion between two mothers of children with Sanfilippo, both of whom have foundations to drive research. When the gene therapy trial came, one mother's son was too far progressed in his disorder. The other mother had one child participate in the trial. They discuss the hope contained in the legacy of their children - inspiring and participating in the science as the pioneers that would help those children who come after.
Video	Michelle & Joe Parents Battens Disease (CLN2)	Clinical Trials: Our expectation is to buy more time	https://courageousparentsne twork.org/videos/cln2-batten -clinical-trials-our-expectatio ns-is-to-buy-more-time	Parents of two sons, one of whom has CLN2, share that their goal with the treatment is to slow down the disease progression and maintain quality of life so as to have more time with their son. Dad stresses the importance of getting information, including from other parents, to set realistic expectations.
Video	Michelle & Joe Parents Battens Disease (CLN2)	Clinical Trials: Not everything is fixed; outcome isn't a cure-all	https://courageousparentsne twork.org/videos/cln2-batten -clinical-trials-not-everything -is-fixed-outcome-isnt-a-cur e-all	Parents of two sons, one of whom has CLN2 and is receiving ERT in a trial, talk about how there are still things the treatment cannot address.