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Palliative Care Needs of Children with Rare Diseases and their Families

IRB Approved Consent

Approved on May 30, 2023

CHILDREN'S NATIONAL MEDICAL CENTER

Center for Translational Science
Washington, DC 20010
(202) 476-5000

Consent/Parental Permission to Participate in a Clinical Research Study and Authorization to Use Protected Health Information

TITLE OF STUDY:	Palliative Care Needs of Children with Rare Diseases and their Families
PRINCIPAL INVESTIGATOR:	Dr. Maureen E. Lyon, PhD, ABPP, Center for Translational Science

Throughout this document, "You" always refers to the person (you or your child) who takes part in the study.

SUMMARY AND KEY INFORMTION

We are inviting you to be part of a research study at Children's National Hospital (Children's National). **Taking part in this study is your choice.** You can choose to take part or you can choose not to take part in this study. You also can change your mind about participating at any time. Whatever choice you make, you will not lose access to your medical care or give up any legal rights or benefits.

To help you decide if you would like to participate, we want you to know why we are doing the study, what you will be expected to do, and the possible risks and benefits of being in the study. This form has information to help you make your choice about whether or not to participate.

The purpose of this research study is, as a caregiver of a child with a rare disease, we want to know what you think your child's health care needs are. We also want to find out about your needs, as the caregiver of your child with a rare disease. We would like to be sure we are meeting the physical, psychological, and spiritual needs of children and their families.

Participants will undergo a baseline screening to determine eligibility for the study. If you are eligible, you will complete baseline surveys via Zoom Telehealth. After surveys are completed, you will be randomized to either our control group or our intervention group. If randomized to the control group, you will have three sessions of our FACE-RARE intervention. There will be three sessions about 2-3 weeks apart. 4 months after completing the baseline visit, there will be a final visit via Zoom Telehealth and the follow-up surveys will be completed. If randomized to the control condition, you will receive treatment as normal, and not have any intervention visits. You will also

complete the follow-up surveys via Zoom telehealth, about 4 months after the baseline visit. Once the follow-up surveys are completed, you'll have the opportunity to receive the intervention. You will not be paid if you do choose to receive the intervention.

Potential risks are minimal. However, some people feel that this is an uncomfortable topic, even though most people do have thoughts about it. If you should find the questions upsetting, we will be available to talk to you and/or make referral to a counselor at Children's National. There is also a risk of a breach of confidentiality. Potential benefits of the study include that you will identify the support needs of your child and yourself, and you will develop an informal action plan to meet your high priority needs.

The alternative to participating would be not to participate in this study and to continue your regular care as previously done.

If you are interested in learning more about this study, please continue reading below. The rest of this form gives you more important information you need to know about the study before you decide if you want to participate. The study doctor or a member of the research team will talk to you about the study and answer all of your questions. We encourage you to discuss this study with your family and anyone else you trust before making your decision. It's important that you have as much information as you need and that all your questions are answered.

Your participation in this research is voluntary.

There will be no penalty or loss of benefits to which you are otherwise entitled if you decide not to be in the study or withdraw from the study later.

This means that:

- You do not have to join the study.
- You may change your mind and stop being in the study at any time.
- We will tell you if we make any important changes to the study or if there are any important new findings so that you can decide if you still want to be in the study.

Why is this research study being done?

You are being asked to be in the study because you are a parent/legal guardian of a minor (≤18 years) child with a rare disease who is unable to participate in health care decision making. The study has two purposes. First, we want to know what you think your child's health care needs are. We also want to find out about your needs, as the caregiver of your child with a rare disease. We would like to be sure we are meeting the physical, psychological, and spiritual needs of children and their families. A second

purpose of this study is to help you and your family talk about your goals of care for treatment for your child, if there were to be a bad outcome, and to make an advance care plan for future treatment that we will share with your doctor. This advance care plan is a document that states what matters most to you for your child's future medical care, including what kind of medical care you may want or may not want for your child. 32 family/patient dyads will be participating/recruited in this study, from Children's National Hospital. The Principal Investigator, or person responsible for this research at Children's Hospital, is Dr. Maureen Lyon. The National Institute of Nursing Research of the National Institutes of Health is paying for this research to be done.

What will happen in this research study?

This study compares the helpfulness of the completion of a caregiver needs assessment and pediatric goals of care action plan with a treatment as usual control. If you decide to participate in the research, here is what will happen. 1. You will complete baseline questionnaires about your caregiving experiences, your physical and psychological quality of life, your religious/spiritual beliefs and practices. We will also collect data on your age, gender, race and ethnicity as well as that of your child. One of our research staff will also have a provider rate your child's functional status or activities of daily living.

2. After you complete the baseline questionnaires, our data base will randomly assign you to either the FACE-Rare intervention arm or Treatment as Usual Control group at a 1:1 ratio. Randomization is like flipping a coin, so you will have an equal chance of being in either group.

3. If you are randomized to the FACE-Rare intervention group you will complete the caregiver needs assessment over the course of two study sessions using the Carer Support Needs Assessment Tool (Sessions 1 and 2) with trained research staff. These interviews are scheduled 2-3 weeks apart. These interviews are not considered standard of care treatment.

- This interview is designed to identify the support needs of your child and yourself as a caregiver to a child with a rare disease.
- You will develop an informal support plan to meet your high priority needs.
- Each of the two sessions will take about 30-45 minutes and will be conducted one to two weeks apart.

3. After completing Sessions 1 and 2, you will return for Session 3 about one week later to complete the pediatric Next Steps: Respecting Choices interview for family caregivers of children with rare diseases. You can bring a support person with you for this visit. These interviews are not considered standard of care treatment.

- This interview will help you to let your doctor know what is important to your child and what matters to you most about your child's future medical care.
- This session will take about 45-60 minutes.

4. The Treatment as Usual group will receive standard of care medical treatment as usual.

- All participants, regardless of group assignment, will complete baseline questionnaires and questionnaires 4 months after baseline. You will complete the same questionnaires from baseline.
- All participants will also complete a Satisfaction Questionnaire and Quality of Communication Questionnaire about how you feel about being in the study. This will happen about week 4.
- All participants will also receive a link or pdf of *The Circle of Care Guidebook for Caregivers of Children with Rare Diseases* (only available online or as a pdf).
- All participants will receive a link to *Conversations Matter: Palliative Care for Children.*
- Families in the control group who express an interest in participating in the intervention may also receive the intervention after their participation after they have completed the final set of questionnaires. Study visits may be scheduled until June 30th.
- Families who chose to participate in the intervention after they have completed their final questionnaires will have the advanced directive created during the additional intervention sessions added to the medical record and sent to their referring physician with their permission.

Study Timeline

Group	Questionaires Week 1	Session 1 Week 2	Session 2 Week 3	Session 3/about Week 4	Questionaires Week 16
Treatment as Usual (TAU) plus FACE-Rare	Baseline	CSNAT	CSNAT	RCI Followed by Satisfaction Questionnaire & Quality of Communication Questionnaire (questionnaires administered by blinded Research Assistant)	About 4 months post-baseline (Questionnaires administered by blinded Research Assistant)
Treatment as Usual (TAU)	Baseline	TAU	TAU	TAU Satisfaction Questionnaire & Quality of Communication Questionnaire	4 months post-baseline (Questionnaires administered by blinded Research Assistant)

				(Questionnaires administered by blinded Research Assistant)	

Key

- CSNAT = The Carer Support Needs Assessment Tool
- RCI = Respecting Choices Interview (end-of-life action plan)
- TAU = Treatment as usual, no study procedures
- There are 6 study visits for FACE-Rare
- There are 3 study visits for Treatment As Usual control

How long will my participation in the research study last?

- *Length of participation*. Please refer to the study timeline listed directly above. The length of participation is approximately four months and consists of either three study meetings (TAU) or six study meetings (FACE-Rare).
- *Regarding a decision to withdraw*. You can decide to stop participating at any time. If you would like to stop participating, all you need to do is tell us. You will not be adversely affected by stopping the study early.
- *Regarding early withdrawal by the PI or sponsor*. We will ask you to drop out of the study if:
    - There are any unexpected side effects
    - Your study doctor and/or the Sponsor of the study thinks it is in your best interest

What are the risks and possible discomforts from being in this research study?
Potential risks are minimal. However, some people feel that this is an uncomfortable topic, even though most people do have thoughts about it. If you should find the questions upsetting, we will be available to talk to you and/or make referral to a counselor at Children's National.

What are the possible benefits from being in this research study?

1. You will identify the support needs of your child and yourself.
2. You will develop an informal action plan to meet your high priority needs.
3. Another benefit for you is that participation may increase your knowledge about end-of-life decision making. It might also make it easier for you to discuss these issues with your family and your child's physician.
4. You will be making a contribution to the care of all children with a rare disease by increasing health care professionals' sensitivity and knowledge of families' support needs and how best to support future medical decision making.

What other choices do I have if I don't want to take part in the study?
The alternative would be not to participate in this study and to continue your regular care as previously done.

Will it cost me anything to take part in the study?
You will not have to pay for study visits or study materials (questionnaires, advance directive) which are required as part of the study. There are no medical costs associated with this study. You will not receive any medications through this study. You will be responsible to traveling to and from the study site or we can conduct the visit at your home or by Telemedicine or telephone, if this is what you would rather do.

Will I be paid for taking part in this study?
Yes. You will receive cash on a "Clincard," which is like a debit card, for your help & your time.

The *Treatment as Usual Group* will receive:
$25 after Baseline questionnaires (30 minutes),
$25 after 4-week assessments (10 minutes),
$25 after 4-month follow-up visit questionnaires,
Totaling $75 for those who complete all 3 study visits.
If a family randomized to the Treatment as Usual Group chooses to also receive the intervention, they will not receive any additional compensation.

The *FACE-Rare group* will receive:
$25 after Baseline questionnaires (30 minutes),
$25 after Session 1 Needs Assessment (30-45 minutes),
$25 after Session 2 review of Action Plan (30 minutes),
$25 after 4-week assessments (10 minutes),
$25 after Session 3 the Respecting Choices conversation (30-45 minutes); and
$25 after completing for 4-month follow-up visit questionnaires,
Totaling $150 for those who complete all 6 study visits.

The Internal Revenue Service requires that any monetary payments totaling $600 or more in a calendar year must be reported for tax purposes.

ClinicalTrials.gov
A description of this clinical trial will be available on http://www.ClinicalTrials.gov, as required by U.S. law. This Web site will not include information that can identify you. At most, the Web site will include a summary of the results. You can search this Web site at any time.

How will you protect my privacy if I take part in this study?

Who will see the information that I give you?

If you decide to take part in this study, only the people working on the study will know your name. They will keep this information on file during the study duration [e.g., so that we can contact you to schedule future appointments]. Your personal information will not be given to anyone besides study staff unless we get your permission in writing or if the law requires it. This information will also be given for regular hospital care, payment, and hospital management activities. We will make every effort to keep your information private, but no one's privacy can be totally guaranteed.

Your medical record is confidential but, just like any medical record, there are some exceptions under state and federal law. These limits of confidentiality are e.g., child abuse/neglect, clear and immediate danger to self or others which we are required to report.

Certificate of Confidentiality

Sometimes people tell us some very personal information about themselves when they participate in a study and it becomes part of their research record. To help us protect your privacy, the investigators have obtained a Certificate of Confidentiality from the Department of Health and Human Services (DHHS).

- With this Certificate, the investigators cannot be forced (for example, by a court order or subpoena) to give information that may identify you in any federal, state or local civil or criminal court, or in any administrative, legislative, or other proceedings.
- It is important that you know that a Certificate of Confidentiality does not stop you or a member of your family from voluntarily giving information to others about yourself or your taking part in this research. You should also know that if an insurer or employer learns about your participation and you give them permission to receive research information about you, we cannot use the Certificate of Confidentiality to keep your information private from them. This means that you must also actively protect your own privacy.
- Finally, it is important that you know that we are not prevented from taking steps to prevent serious harm to you or to others. If we learn that you or someone else is harming you or others around you, we may be required by law to report this to the police or a social services agency to get emergency help if it is needed.

There are some government agencies or other groups that may check records that identify you without your permission. They might review the records of this study and your medical records to make sure we are following the law and protecting the children in the study. The agencies or groups who might see these records are the Office for Human Research Protections, National Institute of Nursing Research/NIH, and the

Institutional Review Board of Children's National Medical Center (the ethics board that reviewed and approved this research study).

The results of this research may be presented at meetings or in publications. You will not be personally identified.

We do not collect any genetic information from you for this study.

A Federal law called “HIPAA” provides additional protections of your medical records and related health information. These protections are described below.

HEALTH INSURANCE PORTABILITY AND ACCOUNTABILITY

In 1996 the government passed a law known as The Health Insurance Portability and Accountability Act (HIPAA). This privacy law protects your individually identifiable health information (Protected Health Information or PHI). The privacy law requires you to sign an agreement so researchers can use or share your PHI for research purposes. This describes to you how information about you may be used or shared if you are in a research study. It is important that you read this carefully and ask a member of the research team to explain anything you do not understand.

I authorize the Principal Investigator, Dr. Maureen Lyon and her research staff to create, access, use, and disclose my PHI for the purposes described below.

Protected Health Information that may be used and shared includes:

- Information that identifies you such as name, home address, email address, telephone number, medical record number, date of birth, and other details about you
- Information that relates to your health or medical condition from your medical records
- Information obtained from the study procedures outlined in this consent form, for example: any medical information we learn from you about your health history and family history
- Questionnaires or surveys you complete
- Interviews conducted with you by members of the research team
- Audio/ video recordings
- Other: ethnicity, education level

The Researchers may use and share my Protected Health Information with:

- The Principal Investigator, other Investigators, Study Coordinators, and all administrative staff in charge of doing work for the study;

- Government agencies that have the right to see or review your PHI including, but not limited to, the Office of Human Research Protections;
- Children's National Medical Center Institutional Review Board;
- Audit Committee of the Children's National Medical Center Institutional Review Board;
- Quality Improvement Program Coordinator and other staff in the Office for the Protection of Human Subjects at Children's National Medical Center.

In addition to the above people and organizations, the Researchers may also use and share my Protected Health Information with:

- The Sponsor of the study and people that the Sponsor may contract with for the study. The name of the Sponsor is the National Institute of Nursing Research of the National Institutes of Health.
- The collaborating organization, Respecting Choices®, a division of C-TAC who developed the advance care planning model and will watch the family interview recordings to assess the fidelity of the interview process;
- The Safety Monitoring Committee (a group of people who examine the medical information during the study)
- The Patient Advocate or Research Ombudsman (person who watches out for your best interest)

Also, your primary physician will be contacted if during the course of the study the researcher learns of a medical condition that needs immediate attention.

Should your health information be disclosed to anyone outside of the study, your information may no longer be protected by HIPAA and this Authorization. However, the use of your health information will still be regulated by applicable federal and state laws.

Storage of PHI in a Database:

We would like to store personal health information collected from you in this study in a database for future research. The database is maintained by Children's National Medical Center, Center for Translational Science.

Please indicate your approval of any or all of the following by initialing next to the statement:

- My personal health information may be stored in the above named database for future analysis related to this study.

  ☐ Yes ☐ No Initials _____

- My personal health information may be stored in the above named database for future analysis related to Palliative Care Needs of Children with Rare Diseases and their Families

  ☐ Yes ☐ No Initials _____

- My personal health information may be stored in the above named database. Researchers may contact me to request my authorization for future studies that are not related to this study or the disease named above.

  ☐ Yes ☐ No Initials _____

- My personal health information may be stored without any of my identifying information for use in other studies of other diseases.

  ☐ Yes ☐ No Initials _____

If you agree to participate in this research study, the research team, the research sponsor (when applicable) and the sponsor's representatives may use Personally De-identified Study Data. The Personally Unidentified Study Data does not include your name, address, telephone, or social security number. Instead, the researcher assigns a code to the Personally Unidentified Study Data. Personally Unidentified Study Data may include your date of birth, initials, and dates you received medical care. Personally Unidentified Study Data may also include the health information used, created, or collected in the research study. The research team or the research sponsor may share the Personally Unidentified Study Data with others to perform additional research, place it into research databases, share it with researchers in the U.S. or other countries, or use it to improve the design of future studies. They may also publish it in scientific journals, or share it with business partners of the sponsor and to file applications with U.S. or foreign government agencies to get approval for new drugs or health care products.

You do not have to sign this Consent/Authorization. If you decide not to sign the Authorization, you will not be allowed to participate in the research study.

After signing the Consent/Authorization, you can change your mind and revoke this Authorization.

- If you revoke the Authorization, you will send a written letter to the Principal Investigator to inform her of your decision.

  Maureen E. Lyon, PhD, ABPP
  Children's National Medical Center
  Center for Translational Science
  111 Michigan Avenue, N.W.
  Washington, DC 20010-2970

- If you revoke this Authorization, researchers may only use and disclose the PHI that was collected for this research study before you revoked the Authorization.
- If you revoke this Authorization, your PHI may still be used and disclosed if you should have an adverse event (unexpected side effect).
- If you change your mind and withdraw the Authorization, you will not be allowed to participate in the study.

You will not be allowed to review the information collected for this research study until after the study is completed. If you are not allowed to review your information during participation in the study, when the study is over you will have the right to access the information.

This Authorization does not expire.

If you have not already received a Notice of Privacy Practices from Children's National Medical Center, you may request a copy and will be given one. If you have any questions or concerns about your privacy rights, you may contact the Children's Hospital Privacy Officer at 301-572-6348.

Whom can I call if I have questions about this research study?

We want you to ask questions about any part of this research study at anytime.

- For questions about the study or the information in this informed consent/parental permission document, call the Principal Investigator, Dr. Maureen E. Lyon at (240) 531-6589.

Whom can I call if I have questions or concerns about my rights as a research study participant?

The Children’s National Office for the Protection of Human Subjects is available to talk with you about:

- Your rights as a research participant
- Your concerns about the research
- A complaint about the research

This is the administration office for the Institutional Review Board, which is a group of doctors, nurses, and non-medical people who review research studies for safety and the protection of people who participate in research. You can call the Office for the Protection of Human Subjects at 301-565-8447.

Children’s National has a research participant and family advocate. The advocate is here to answer your questions or concerns about taking part in this research. The advocate does not work for the doctors who are doing this research and is not paid by the researchers. The advocate is here only to help and protect you during any research.

You may contact the research advocate at any time. This can be done before you decide to take part in the research, during the study, or even after you finish the study. You can contact the research advocate at 202-476-5586 or by email at RSA@childrensnational.org. In urgent situations the research advocate and pediatric ethics program team can be reached at the pager number: 202-259-2082.

CONSENT/PARENTAL PERMISSION:

- I am the study participant or I am authorized to act on behalf of the participant.
- I have read this consent form or had it read to me.
- I have been invited to take part in a research study. I was told why the research is being done and how long my participation in the study is expected to last. I was told about what will happen in the study and if there are any procedures or drugs that are experimental.
- I was told that taking part in this research is voluntary. I also was told that I can decide not to take part or stop being in it at any time without any penalty to me or any change to the quality of care I receive at Children's National.
- I was told about the risks and possible discomforts of taking part in this research study. I was also informed if there are any possible benefits to me if I am in this study.
- I have been given the chance to ask questions about the study, and my questions have been answered. If I have questions later, I can ask one of the people listed in this form.
- I agree to take part in this research study.
- I will receive a copy of this Informed Consent/Parental Permission form to keep.

Signature of Parent(s)/Guardian for participant under the age of 18 years

Printed Name of Participant: _____

Printed Name of Parent/Guardian: _____

Signature of Parent/Guardian: _____

Date and Time:_____ a.m. / p.m. (circle one)

Signature of adult participant (18 years of age and older)

Printed Name of Participant: _____

Signature of Participant: _____

Date and Time: _____ a.m. / p.m. (circle one)

AFFIDAVIT OF PERSON OBTAINING CONSENT / PARENTAL PERMISSION:

I certify that I have explained to the above individual(s) the nature and purpose of the study, potential benefits, and possible risks associated with participation in this study. I have answered any questions that have been raised.

Printed Name of Person Obtaining Consent: _____

Research Role: _____

Signature: _____

Date and Time: _____ a.m. / p.m. (circle one)

Signature of Witness (if applicable)

Printed Name of Witness: _____

Witness's Signature: _____

Date and Time: _____ a.m. / p.m. (circle one)