



EXPLORING TREATMENT OPTIONS FOR RARE DISEASE: PEDIATRIC PALLIATIVE CARE

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Introduction

There are no drug treatments available for approximately 95 percent of the nearly 7,000 identified rare diseases. There is, however, a way to improve the quality of life of children living with serious and life-limiting illnesses: pediatric palliative care. This is provided by an interdisciplinary team, including physicians, nurses, and social workers, who work closely with the family and primary care providers.

Palliative care is often misunderstood. People associate it with end-of-life care, hospice care, or “giving up” – especially when facing a serious health challenge. But this is not the case. This toolkit addresses this misconception, providing an introduction on this care option, how it benefits the family, and how to gain access and learn more.



SECTION 1:



WHAT IS PEDIATRIC PALLIATIVE CARE?

When a child is seriously ill, everyone in the family is affected. Caring for a child with a life-threatening illness can be overwhelming for the parents and isolating for everyone in the family. **Pediatric palliative care** is an *added* layer of support that can provide relief from the symptoms, pain, and psychological and emotional stress associated with the illness—regardless the diagnosis and the plans for curative treatments. And pediatric palliative care teams work closely with a child’s existing team of providers to provide the best care possible.

Pediatric palliative care can benefit children with all serious illnesses, including genetic disorders, neurologic disorders, heart and lung conditions, and advanced cancers.

Pediatric palliative care’s goal is simple: to improve the quality of life for the child and the family. In short, it helps the child and the family to have the physical, emotional, spiritual and mental strength to carry on with daily life and provide the best quality of life for the patient throughout the illness journey. It does this by:

- Relieving the child’s disease symptoms, such as pain, shortness of breath, fatigue, constipation, nausea, loss of appetite, and difficulty sleeping.
- Working with the parents and family members to identify their care goals and values.

“Palliative care gives families the courage they need to make difficult decisions and face the unknown,” says Tracy Larson-Benvenuti, MSW, LICSW, Director of the Pediatric Palliative Care Home-Care in Worcester, Massachusetts.” Palliative care providers help the family to reframe their ideas of ‘hope’ and gain control of a senseless situation. When the goals of care shift from cure to comfort, palliative care providers are able to assist families to define their quality of life goals and their ‘new’ ideas of hope.”

- Helping communicate and coordinate the care between the medical team and the parents so families are better able to make choices that are in line with their values, traditions, and culture.
- Giving parents the encouragement and skills to talk with their affected child and other children about illness.
- Providing parents with resources around self-care and coping, and emotional and spiritual support.
- Providing guidance and navigation through the healthcare system.
- Providing resources to other members of the family’s community—such as teachers and friends—so they are informed of the family’s needs and situation.

“The palliative care team that worked with our family during our son Benjamin’s illness helped us develop a philosophy of care that would help guide us in the decision making process,” says Abby Rogers, mother of a child with a rare disease. “We were able to frame difficult choices and decisions based on this plan. We felt supported and strengthened by considering these potential choices prior to actually having to make them. It

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helped to lessen our anxiety about the road ahead and let us focus on enjoying our child in the moment which enhanced his quality of life as well as ours.”

The Difference Between Palliative Care and Hospice

Palliative care can benefit anyone with a serious illness at any age and at any stage of illness. It is not dependent on prognosis or whether the condition can be cured.

Hospice care is an extension of pediatric palliative care, and often, palliative care is delivered through hospice providers who are trained in helping families realize their quality of life goals for their child.

But palliative care is **not** hospice care. People who are officially receiving hospice care must have a prognosis of less than 6 months and usually are no longer able to also receive curative treatment for the underlying disease.

The Difference Between Adult and Pediatric Palliative Care

Because the causes and treatment of illness and death for children are so different from those of adults, pediatric palliative providers need to have a different knowledge base from those caring for adult patients. Children have different needs than adults and will have different reactions to illness depending on their age and developmental stage. They also are growing and developing as they go through an illness. Therefore, all specialized medical care, including palliative care, must be tailored to meet the needs of infants, children, and adolescents.

The main differences are:

- While serious illness in anyone is upsetting, it is particularly so when it occurs in children. The notion that a child may die goes against the natural order of things and thus presents additional stresses to the family.
- Medical decisions for young children are usually made by their caregivers, while adult patients may make their own decisions.
- Pediatric palliative care can also involve other specialists not typically used for adults. These may include a play therapist, child life therapist, and/or child behavioral specialist.

Pediatric palliative care can be provided along with curative treatments when those are available and is appropriate at any stage of an illness. Ideally, palliative care begins early in the disease journey, so families have this extra layer of support throughout their child’s continuum of care.

“Part of our job as palliative care clinicians, when there is no hope for a cure, is to help families and their child to find the other hopes that they can achieve, and to identify and nurture what is most important to them, what else it is they wish for,” says Dr. Patricia O’Malley, Director of Pediatric Palliative Care, Massachusetts General Hospital for Children. “Even when we cannot change the ultimate outcome, we can almost always find ways to improve the journey. There is always hope.”

Anticipatory grief may begin at the time of diagnosis, when the family learns that the future is not as they expected. When this grief and shift in expectations are addressed, they can be managed in a way that helps the entire family to cope with the new reality of their life.

SECTION 2: THE PALLIATIVE CARE TEAM



Pediatric palliative care is **interdisciplinary** and may involve:

- Physicians
- Nurses
- Social workers
- Child life specialists
- Home health aides
- Chaplains
- Psychologists
- Nutritionists
- Specialists such as massage therapists and music therapists
- Grief counselors

The team works together with a child's primary physicians as an *added* layer of support. By blending palliative care with curative treatments, they provide the child and family with the most complete medical care possible.

"The pediatric palliative care team strives to form a relationship with the child and with the family members to understand and learn what is most important to each of them," says Janet Duncan, MSN, CPNP, nurse practitioner of the Pediatric Advanced Care Team at Boston Children's Hospital. "As we help families determine what treatment options best fit with their values, beliefs, and goals, we advocate for that. We strive for optimal symptom management and making each day as good as it can be."

Pediatric palliative care providers help parents and children have conversations about difficult subject. Their top priority is family-centered care, not disease-directed care, and thus they place the needs and priorities of the family at the fore.

Palliative Care in the Hospital

Children being treated for life-threatening illness can benefit from the additional care **palliative care specialists** provide. Ideally, the pediatrician and/or the disease specialist working with the family (for example, oncologist, neurologist, endocrinologist, or cardiologist) refer the family to the pediatric palliative care team, which works in tandem with the health-care providers treating the disease.

Families can also ask for a palliative care consultation themselves. Asking for this does not mean that the established team of specialists will no longer be part of the child's care. Palliative care specialists will partner with the family and child's existing team.

Families often first meet the pediatric palliative care team at the hospital or clinic appointment. Many teams will continue to offer care and support via telephone and home visits once the child is at home, and follow-up visits can take place in either of these locations.

Palliative Care in the Home

A child's hospital team may or may not be able to provide home visits. Some hospital teams have a standing relationship with agencies that provide in-home services. In some states, such as Massachusetts, there is a **state agency network** (<http://www.mass.gov/eohhs/gov/departments/dph/programs/family-health/pediatric-palliative-care.html>) of home services that may provide services separate from the hospital team. Families should research if these types of services are available in their state.

"Medically, the team was always a phone call away," says Cindy Cook, mother of a child with a rare disease. "I remember one time, when Jonathan was moaning from a leg injury, I could not figure out what was wrong. I took off a leg brace and he had developed

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a small pressure sore from the brace. I was terrified. I called our palliative care nurse frantic, and she was at our house within an hour. She cleaned the wound, had the doctor prescribed medications, and showed me what to do. Jonathan was healed within a week. If I did not have palliative care, that would of required a trip to the doctor or hospital—out in the cold, with a medically fragile child, making him susceptible to other illnesses.”

Home-based programs may provide another layer of medical support, or may focus more on psychosocial support. The home-based team is also interdisciplinary, and it may include a physician, nurse, chaplain, social worker, massage therapist, music therapist, and child life specialist who work with the child and any siblings.

SECTION 3:

OBTAINING ACCESS TO PALLIATIVE CARE



Families that think palliative care could help them, their children should request it. They can ask their child's pediatrician or specialist about adding palliative care to the treatment team, and ask their provider to make a referral for palliative care services.

When Families Are Told, "Palliative Care is Not for You"

It is not uncommon for parents, when asking their healthcare provider for palliative care services, to be told that their family situation isn't ready for this type of care because it has not transitioned to end-of-life care. This is a big **misconception** for palliative care. If faced with this response, here are some important points to explain to get the needed care needed:

- Most children receiving palliative care are not actively dying, but are rather actively living with a condition that may shorten their lives, and therefore, they may receive palliative care for years.
- Palliative care ensures comfort at every stage of the illness and should not be equated with end of life. Palliative care helps to make the quality of each day better, regardless of the amount of time the child has.
- Palliative care can be used in conjunction with curative therapies and is meant to complement, not replace, curative treatments.

- Palliative care involves reviewing the family's care goals and values again and again, as the disease changes/progresses. It is a process and philosophy of care, not an end in-and-of itself.

"As healthcare becomes ever more complex and treatment options increase, it is challenging for parents of children with serious illnesses to make difficult decisions about their children's care," says Sandy Clancy, mother of a son with a rare disease and Program Manager and Patient Advocate, Pediatric Palliative Care at Massachusetts General Hospital for Children. "Palliative care practitioners communicate with families to enable them to keep their children's quality of life at the center of decision making."

Locating Palliative Care Programs

To find a hospital in your area that offers a palliative care program, go to **Get Palliative Care's Palliative Care Provider Directory of Hospitals** (<http://getpalliativecare.org/providers/>) and search by state and city. The hospital can then refer you to those programs in the area that provide in-home palliative care for children.

Additional resources to find a pediatric palliative/hospice program within your area include:

- **Hospice Foundation of America** (<http://www.hospicedirectory.org>): Use this organization's Hospice Directory to locate a hospice. Also learn more about this type of care through its Frequently Asked Questions or emailing them a question directly.
- **National Hospice & Palliative Care Organization** (<http://www.nhpco.org/resources/choosing-hospice>): The National Hospice and Palliative Care Organization

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(NHPCO) is the largest nonprofit membership organization representing hospice and palliative care programs and professionals in the United States. This resource provides regional lists to help people searching for a program within their area.

What are the Costs?

Most insurance plans, including Medicare and Medicaid, cover palliative care treatment. Families should check with their insurance company or HMO to determine their coverage as many offer palliative care benefits.

Medicare, a government insurance program, covers people 65 years or older, people who are younger who have chronic conditions, and people at any age who have kidney failure.

Medicare Part B, a non-compulsory option that you must pay for, may offer palliative coverage, depending on the treatment. Medicare coverage is explained further on the **U.S. Department of Health and Human Services** website (<http://www.medicare.gov/>).

Medicaid, a health insurance plan for those with low income, may cover some palliative care treatments and medications, including visits from doctors. But it does not use the term “palliative,” so coverage is provided by standard Medicaid benefits. The palliative care provider (the organization offering the services) will bill Medicaid for services provided, but be sure to research what co-pays or fees patients may be asked to pay.

For specific questions about insurance coverage of palliative care services, ask a physician, social worker, another member of a palliative care team, a member of the hospital’s financial services department or the health plan directly.



APPENDIX: A PERSONAL REFLECTION

Blyth Lord, Courageous Parents Network

“The world was broken and the three of us were falling into its mouth.” – The Still Point of the Turning Word, Emily Rapp

I love this quote because it captures so perfectly the experience parents have when their child is diagnosed with a rare and life-threatening illness. I fell through the world when my daughter Cameron was diagnosed at age 6 months with infantile Tay-Sachs disease, a genetic disease that affects the central nervous system and for which there is still no treatment or cure. Tay-Sachs is progressive and always fatal. My daughter died at the age of 2.

My family spent the first few months reviewing the ongoing research to see if there was any hope for treatment. We talked to scientists in New York, Massachusetts, and California, but there was nothing hopeful for our daughter. So where was the hope going to come from? We did have each other and an amazing extended family and an older daughter who was 2 at the time. But these people knew nothing about disease and couldn't help us navigate the scary road of choices and care that lay ahead. We felt vulnerable and exposed and afraid that we might make a mess of things and be riddled with regret. We had one job to do for our daughter: to give her the best life she could have, for as long as she could have. Who was going to help us do that?

The help came from our daughter's pediatrician who practiced palliative care and ushered us slowly into the framework of thinking and decision-making that is central to palliative care work. He asked us what our goals of care were for Cameron and what level of invasive treatments we would tolerate for her. He helped us

- Find a child psychologist with whom we could talk about how to explain things to our older daughter
- Manage Cameron's grand mal seizures at home, by ourselves, so we could keep her out of the hospital
- Find a pain specialist with whom we could talk about managing Cameron's possible discomfort towards her end of life
- Complete the DNR form in the event we would need it

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He didn't do this all at once of course. There wasn't just the "one" conversation; there were multiple ones that happened over the course of Cameron's life and the disease trajectory. Most rare diseases move slowly enough that parents and those patients who are cognitively capable have the "gift of time" to think things through before the crises break. Indeed, one of the most reassuring things he told us after delivering Cameron's diagnosis was, "You don't have to figure this out all at once. Go home, and when you're ready, we'll start talking about what Cameron will need and how we can help meet these needs." Under his care, we experienced how palliative medicine is precisely about making use of time to anticipate needs and make thoughtful decisions with the information at hand. Even then, we never felt our agency as parents was being hijacked by the medical machine.

Reflecting back on our daughter's time with us, we can say that we gave Cameron a full life, with a beginning, a middle, and a gentle end. We have very few regrets, and none of any impact. And this is perhaps the most important thing that palliative medicine offers: the empowerment and agency to say what is it you want for the patient's care and life beyond the disease itself. We didn't have to say YES to every intervention available. We didn't have to surrender decision-making to the specialists. Our daughter Cameron's story was in our hands, and we helped it be a good one, and that has made all of the difference.

RESOURCE GUIDE



Learning More About Palliative Care

Courageous Parents Network (<http://courageousparentsnetwork.org/>): This organization's mission is to support parents and families of children living with serious illness. They provide coping resources, parent-to-parent support, information on pediatric palliative care, and a voice to those whose children are facing end-of-life.

Get Palliative Care (<http://getpalliativecare.org/>): Provided by the Center to Advance Palliative Care, this site provides clear, comprehensive palliative care information for people coping with serious, complex illness.

Partnership for Palliative Care (<http://restoringqualityoflife.org/what-is-palliative-care>): This nonprofit organization focuses on palliative care service expansion, research, advocacy, and public education. The overarching mission of the Partnership's work is to raise public awareness of palliative care so more people living with chronic disease and serious illness can benefit from its effects.

The Week's "Everything You Need to Know About Palliative Care" (<http://theweek.com/article/index/236639/everything-you-need-to-know-about-palliative-care>): This article, written by Bill Frist, provides additional information on this type of care and how it can benefit those with serious, chronic illnesses and their families.

Locating Palliative Care Programs

Get Palliative Care (<http://getpalliativecare.org/providers/>): Provided by the Center to Advance Palliative Care, this site has a Palliative Care Provider Directory that can help you or a loved one locate a hospital that provides palliative care.

National Hospice & Palliative Care Organization (<http://www.nhpco.org/resources/choosing-hospice>): The National Hospice and Palliative Care Organization is the largest nonprofit membership organization representing hospice and palliative care programs and professionals in the United States. If you are searching for a program within your area, this resource provides regional lists.

RESOURCE GUIDE



Talking with Doctors

Becoming an Empowered Patient: A Toolkit for the Undiagnosed (<http://globalgenes.org/toolkits/becoming-an-empowered-patient-a-toolkit-for-the-undiagnosed/introduction-2/>): This resource provides additional information on how to speak to doctors, how to prepare for appointments, and questions to ask.

Caring Connections' "Talking to Your Child's Doctor: When Your Child Has a Serious Illness" (<http://www.caringinfo.org/i4a/pages/index.cfm?pageid=3336>): This resource provides practical advice for readers to prepare them before visiting their children's doctors.

Courageous Parents Network's "A Framework for the Difficult Decisions" (<http://courageousparentsnetwork.org/parent-coping-resources/a-framework-for-the-difficult-decisions/>): This resource provides families with some questions to consider and discuss with the medical team helping their children.

Seattle Children's Hospital's "Decision Making Tool" (<http://www.seattlechildrens.org/clinics-programs/palliative-care-consultation/>): This tool was developed to help families talk about what is important to them and their child. It also helps make sure that the child's healthcare team understands what a family wants, how to work with them, and how best to care for their child. The tool helps parents, their family, and their child think about the care they want.

RESOURCE GUIDE



Videos for Further Learning

Courageous Parents Network's "Palliative Care Is the Beginning of Finding Your Way" (<https://www.youtube.com/watch?v=Q149efLLT3I&feature=youtu.be>): A pediatric palliative care doctor talks about some of the practical and bigger things the palliative care team does.

Courageous Parents Network's "Value of Home-Based Palliative Care" (<https://www.youtube.com/watch?v=wGRwcuDm2Qg&feature=youtu.be>): A palliative care social worker describes home-based palliative care services and the benefits of being able to see a family and the family dynamics in a non-clinical setting.

Courageous Parents Network's "Provide Parents with Hope" (<https://www.youtube.com/watch?v=locW4DTDUS0&feature=youtu.be>): A palliative care social worker talks about the importance of hope and helping parents find hope all along their child's illness journey.

Courageous Parents Network's "Decision Making & Palliative Care" (https://www.youtube.com/watch?v=NS1wDRPb_AI&feature=youtu.be): Grief counselor, Nancy Frumer Styron, discusses the range of decisions that parents face right from the beginning: practical, logistical, financial, and familial.

Get Palliative Care's "What Palliative Care Is and Why It Matters" (<https://www.youtube.com/watch?v=C2zfnCaE4dw>): Palliative care specialists, advocates, and patients discuss what palliative care is and how it is beneficial to those facing serious illness.

Get Palliative Care's "Palliative Care: You are the Bridge" (<https://www.youtube.com/watch?v=IDHhg76tMHc>): This video animation compares palliative care to the foundation of a bridge. While illness may weaken the foundation, the palliative care team provides a stronger layer of support.



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