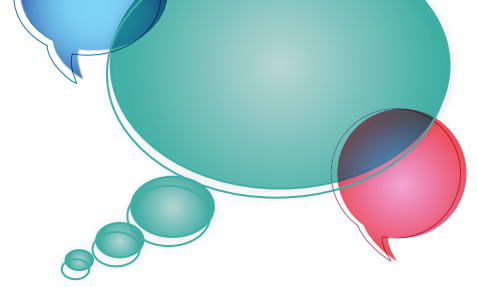


CYSTINOSIS CONVERSATIONS

Let's Talk About Cystinosis Care



How to Use This Clinician Tool

Treatment adherence is a challenging topic to address during clinic visits. This tool was developed to initiate and support ongoing conversations between clinicians, patients with cystinosis, and caregivers about adherence and remaining engaged in care.

- 1 This tool contains 2 handouts, which are tailored to teen/young adult and adult patients, along with additional clinician information to support in-clinic discussions
- 2 Each patient handout contains a brief questionnaire to track progress over time, a guided goal setting activity, and additional information about cystinosis
- 3 The handouts are intended to be used during routine clinic visits and taken home by patients and/or their caregivers

**VIEW AND PRINT THE HANDOUT
FOR YOUNG ADULT PATIENTS**

**VIEW AND PRINT THE HANDOUT
FOR ADULT PATIENTS**

Information for Clinicians Exploring Adherence in Cystinosis

Without adequate cystinosis management, cystine buildup leads to irreversible damage throughout the body, often progressing without obvious symptoms.¹⁻³ Because of the progressive nature of cystinosis, lifelong adherence to multiple medications, including immunosuppressants, cystine-depleting therapy, and supportive treatments for extrarenal complications, is required.^{3,4}

In a self-reported survey representing 61% of the cystinosis population in Spain, adherence tends to decline in adolescence and adulthood, placing these groups at risk for worse outcomes.^{2,5,6,a}

89%

**OF INDIVIDUALS YOUNGER
THAN 11 YEARS OF AGE**
took all their daily CDT doses^{5,a}

56%

**OF INDIVIDUALS 11 YEARS OF AGE
AND OLDER** took all their daily
CDT doses^{5,a}

Although causes of nonadherence are complex and unique to each patient, factors that may impact individuals with cystinosis include^{4,7}:

- Polypharmacy and dosing frequency
- Medication intolerance
- Side effects
- Limited understanding of disease progression
- Challenges during the transition to adult care
- Treatment fatigue

^aData from cysteamine adherence survey completed by patients (15/34) or their caregivers (19/34).⁵

Individuals with cystinosis may have challenges with working memory, behavior initiation, task planning, and organization. Living with a chronic disease can also evoke a fatalistic outlook and lead to psychological impacts, which clinicians should consider as they monitor for and support care engagement and adherence.^{6,8,9}

RESEARCH HAS SHOWN THAT INSTILLING AND MAINTAINING HOPE FOR THE FUTURE

is an important motivator for maintaining patient engagement in care⁹



Supporting Adherence and Care Engagement

The health care team can support adherence and help patients control their disease by^{7,10}:

Acknowledging that lifelong adherence is difficult and that nonadherence may be due to factors outside of the patient's control

Taking time to **understand the patient's unique adherence challenges** and regularly assessing barriers

Actively involving the patient in their care, including setting goals, planning, and problem-solving to remove barriers and overcome challenges



SUGGESTED EVIDENCE-BASED APPROACHES TO SUPPORT DISCUSSIONS WITH PATIENTS INCLUDE^{5,11-13}:

- 1 Encouraging self-management skills in adolescence and young adulthood
- 2 Routinely assessing adherence with laboratory testing (eg, drug assays, white blood cell cystine level testing)
- 3 Exploring medication-related challenges and simplifying medication regimens where possible
- 4 Identifying cues/visual reminders (eg, cell phone alarms, medication calendar, pill organizer, and planning medication timing around daily activities like brushing teeth)
- 5 Reinforcing medications with dose administration aids or reminder tools
- 6 Using the teach-back method to assess understanding when providing counseling/education
- 7 Linking patients with support for logistical and financial challenges

See the following resources for additional information

Resources for Clinicians



[UnderstandingCystinosis.com](https://www.understandingcystinosis.com)



[GotTransition.org](https://www.gottransition.org)



[WBCkit.com](https://www.wbckit.com)

Resources for Patients



[visithangout.com](https://www.visithangout.com)



[CystinosisUnited.com](https://www.cystinosisunited.com)

FOR TEENS
AND YOUNG
ADULTS
LIVING WITH
CYSTINOSIS

Cystinosis

CHECK-IN FOR YOUNG ADULTS

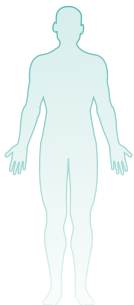


“Taking my medications can be a struggle, but I take them so I can do the things I want to do.”
– Young Adult With Cystinosis

Tracking Your Progress

For each statement below, check the box that best describes how you feel. There are no right or wrong answers; it's about **your real feelings**.

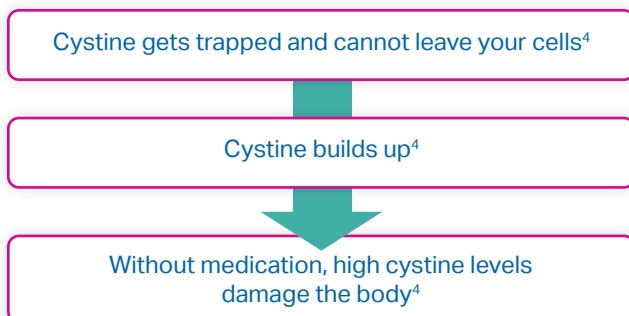
	NO, I DON'T	NO, BUT I WANT TO	YES, BUT I DON'T ALWAYS GET IT RIGHT	YES, I DO
1. I know which medications I take, what they're for, and when to take them	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
2. I take my medications as prescribed by my doctor	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
3. I feel comfortable talking to my doctors and nurses about the challenges I have with taking my medications on time, every time	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
4. I keep track of my cystine levels and know my target number	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
5. I have people in my life to lean on when I'm going through hard times	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>



LET'S FOCUS ON YOUR WHOLE BODY

It's important to manage what's going on in your body *today* in order to be the healthiest you can be *tomorrow*.^{1,2}

When you have cystinosis, an amino acid called cystine builds up and harms the cells in different parts of your body over time. You may not be able to see or feel the damage that is happening until you feel bad or have symptoms.^{2,3}



The goals of cystinosis care are to lower your cystine levels and slow down damage in your body.⁴⁻⁶

Managing your cystinosis includes⁷:

- 1 Keeping your kidneys healthy
- 2 Caring for other organs that could be damaged over time, like your eyes and muscles
- 3 Lowering cystine levels with cystine-depleting therapy (CDT)

Cystine level testing helps your doctors and nurses see how your cystinosis care is working and adjust your medication dosage, if needed.⁸

Let's Talk

CAN YOU THINK OF 3 THINGS **YOU** DO TO TAKE CARE OF YOUR CYSTINOSIS?



TAKING YOUR CDT ON TIME EVERY TIME

is important for keeping your cystine levels controlled and limiting damage to the body.^{3,9}

PAVING YOUR OWN WAY

Your doctors and nurses want to help **YOU** take charge of your cystinosis care by taking small steps toward greater independence in adulthood.¹⁰

Your journey to adulthood is *personal*, and you get to decide how it goes. Your family, doctors, and nurses are there to support you along the way.¹⁰

« YOU MIGHT TRY¹⁰

SETTING UP MEDICATION REMINDERS
so your parents don't have to remind you as often

MAKING PLANS FOR YOUR FUTURE and thinking about what you want your life to be; write things down and see how your ideas grow!

SCHEDULING
your own medical appointments with the help of a parent

ANSWERING AS MANY OF YOUR DOCTOR'S QUESTIONS as you can and asking your doctor follow-up questions during the visit

CALLING THE PHARMACY or using their online portal to refill your prescriptions with the help of a parent



JUGGLING FAMILY, FRIENDS, SCHOOL, HOBBIES, AND CYSTINOSIS IS NO SMALL TASK. TAKE IT ONE STEP AT A TIME. **YOU'VE GOT THIS!¹⁰**



READY

- Planning and talking about your goals can motivate you to tackle challenges and keep growing
- Think about something you'd like to work on to support your health and well-being. Why is this important to you?



SET

- This is your time to plan! How can you set your goals in motion?
- Start small and pick something that lets you measure and celebrate your success. What smaller goals would help you reach your long-term goal?



GO

- Write down your goals and next steps
- Finally, set up a time to check in with your doctors and nurses (at the next clinic visit, phone call, or virtual visit)

**This is YOUR care!
Get involved!**

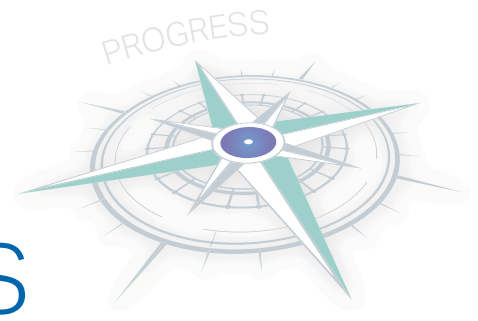
GOALS

References

1. Beinart N, Hackett RA, Graham CD, Weinman J, Ostermann M. Mood and illness experiences of adults with cystinosis. *Ren Fail.* 2015;37(5):835-839. 2. Wilmer MJ, Schoeber JP, van den Heuvel LP, Levtchenko EN. Cystinosis: practical tools for diagnosis and treatment. *Pediatr Nephrol.* 2011;26(2):205-215. 3. Elmonem MA, Veys KR, Soliman NA, van Dyck M, van den Heuvel LP, Levtchenko E. Cystinosis: a review. *Orphanet J Rare Dis.* 2016;11:47. 4. Gahl WA, Thoene JG, Schneider JA. Cystinosis. *N Engl J Med.* 2002;347(2):111-121. 5. Brodin-Sartorius A, Tête MJ, Niaudet P, et al. Cysteamine therapy delays the progression of nephropathic cystinosis in late adolescents and adults. *Kidney Int.* 2012;81(2):179-189. 6. Nesterova G, Gahl W. Nephropathic cystinosis: late complications of a multisystemic disease. *Pediatr Nephrol.* 2008;23(6):863-878. 7. Ariceta G, Camacho JA, Fernández-Obispo M, et al. Cystinosis in adult and adolescent patients: recommendations for the comprehensive care of cystinosis. *Nefrologia.* 2015;35(3):304-321. 8. Langman CB, Barshop BA, Deschênes G, et al. Controversies and research agenda in nephropathic cystinosis: conclusions from a "Kidney Disease: Improving Global Outcomes" (KDIGO) controversies conference. *Kidney Int.* 2016;89(6):1192-1203. 9. Levtchenko EN, van Dael CM, de Graaf-Hess AC, et al. Strict cysteamine dose regimen is required to prevent nocturnal cystine accumulation in cystinosis. *Pediatr Nephrol.* 2006;21(1):110-113. 10. Cystinosis Research Network. Transition guide for teens and young adults with cystinosis and their families. Accessed October 12, 2022. https://cystinosis.org/wp-content/uploads/2019/01/FINAL_CRN_Transition_TeensAdults-3.pdf

Cystinosis

CHECK-IN FOR ADULTS



“I’m proud of everything that I’ve done...if you take your medicine and care for your body, you can aspire for these things [that you want to do].” – *Adult With Cystinosis*

Tracking Your Progress

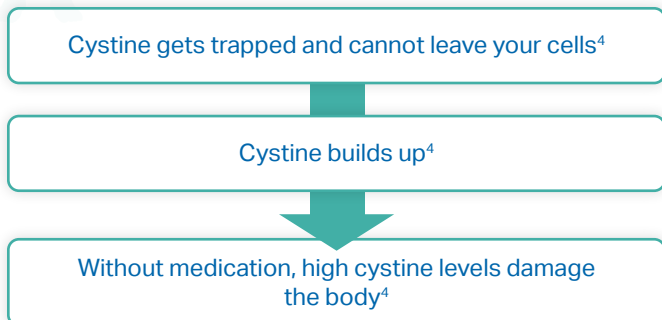
For each statement below, check the box that best describes how you feel. There are no right or wrong answers; it’s about **your real feelings**.

	NO, I DON'T	NO, BUT I WANT TO	YES, BUT I DON'T ALWAYS GET IT RIGHT	YES, I DO
1. I know which medications I take, what they're for, and when to take them	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
2. I take my medications as prescribed by my doctor	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
3. I understand how to refill my prescriptions and don't currently experience issues with my insurance or pharmacy	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
4. I keep track of my white blood cell cystine level results and know my target number	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
5. I have people in my life to lean on when I'm going through difficult times	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

LET'S FOCUS ON YOUR WHOLE BODY

It's important to manage what's going on in your body *today* in order to be the healthiest you can be *tomorrow*.^{1,2}

If cystine is allowed to build up over time, it harms cells and organs throughout the body. You may not be able to see or feel the damage that high cystine levels cause. Often, you may not realize damage is occurring until you have symptoms.^{2,3}



The goals of cystinosis management are to keep cystine levels low and to slow down or limit damage to the body.⁴⁻⁶

Let's Talk

WHICH PARTS OF YOUR CYSTINOSIS CARE ARE CURRENTLY GOING WELL?

Managing your cystinosis includes⁷:

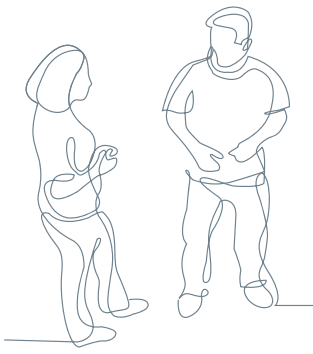
- 1 Maintaining your kidney health
- 2 Caring for organs and body systems that could be damaged over time, like your eyes and muscles
- 3 Lowering cystine levels with cystine-depleting therapy (CDT)

Cystine level testing helps your doctors and nurses see how your cystinosis care is working and adjust your medication dosage, if needed.⁸



TAKING YOUR CDT ON TIME EVERY TIME

is important for keeping your cystine levels controlled and limiting damage to the body.^{3,9}



LIVING YOUR BEST LIFE

Adulthood comes with new challenges, including transitioning to doctors and nurses who are focused on the care of adults and taking more responsibility for your cystinosis management.¹⁰

At the same time, you might also be navigating big life changes, like leaving home, starting a new job or educational path, managing your own finances and insurance, and forming new relationships.¹⁰

It's normal to go through ups and downs as you navigate adulthood. Lean on the people in your life to support YOU. Your support group can cheer you on when you celebrate success and provide encouragement when you're going through something difficult.¹⁰

WHO IS ON YOUR CYSTINOSIS SUPPORT TEAM¹⁰



FRIENDS AND FAMILY



DOCTORS AND NURSES



THE CYSTINOSIS COMMUNITY



MAKE A LIST OF YOUR UPCOMING LIFE CHANGES.

Which are you most excited about? What are you nervous for, and why?

READY

- Planning and visualizing your goals can motivate you to tackle challenges and identify areas for growth
- Think about something you'd like to work on to support your health and well-being. Is there anything you can improve on? Why is this important to you?

SET

- This is your time to plan! How can you set your goals in motion?
- Start small and pick something that lets you measure and celebrate your success. What smaller goals would help you reach your long-term goal?

GOALS

GO

- Write down your goals and next steps
- Finally, set up a time to check in on your progress (eg, at the next clinic visit, phone call, or virtual visit)

This is YOUR care. Get involved!

No one is going to get it right every time. Don't give up if it doesn't go according to plan.

THE GOAL IS PROGRESS OVER PERFECTION.



References

1. Beinart N, Hackett RA, Graham CD, Weinman J, Ostermann M. Mood and illness experiences of adults with cystinosis. *Ren Fail.* 2015;37(5):835-839.
2. Wilmer MJ, Schoeber JP, van den Heuvel LP, Levchenko EN. Cystinosis: practical tools for diagnosis and treatment. *Pediatr Nephrol.* 2011;26(2):205-215.
3. Elmonem MA, Veys KR, Soliman NA, van Dyck M, van den Heuvel LP, Levchenko E. Cystinosis: a review. *Orphanet J Rare Dis.* 2016;11:47.
4. Gahl WA, Thoene JG, Schneider JA. Cystinosis. *N Engl J Med.* 2002;347(2):111-121.
5. Brodin-Sartorius A, Tête MJ, Niaudet P, et al. Cysteamine therapy delays the progression of nephropathic cystinosis in late adolescents and adults. *Kidney Int.* 2012;81(2):179-189.
6. Nesterova G, Gahl W. Nephropathic cystinosis: late complications of a multisystemic disease. *Pediatr Nephrol.* 2008;23(6):863-878.
7. Ariceta G, Camacho JA, Fernández-Obispo M, et al. Cystinosis in adult and adolescent patients: recommendations for the comprehensive care of cystinosis. *Nefrologia.* 2015;35(3):304-321.
8. Langman CB, Barshop BA, Deschênes G, et al. Controversies and research agenda in nephropathic cystinosis: conclusions from a "Kidney Disease: Improving Global Outcomes" (KDIGO) controversies conference. *Kidney Int.* 2016;89(6):1192-1203.
9. Levchenko EN, van Dael CM, de Graaf-Hess AC, et al. Strict cysteamine dose regimen is required to prevent nocturnal cystine accumulation in cystinosis. *Pediatr Nephrol.* 2006;21(1):110-113.
10. Cystinosis Research Network. Transition guide for teens and young adults with cystinosis and their families. Accessed October 12, 2022. https://cystinosis.org/wp-content/uploads/2019/01/FINAL_CRN_Transition_TeensAdults-3.pdf

References

1. Gahl WA, Balog JZ, Kleta R. Nephropathic cystinosis in adults: natural history and effects of oral cysteamine therapy. *Ann Intern Med.* 2007;147(4):242-250.
2. Elmonem MA, Veys KR, Soliman NA, van Dyck M, van den Heuvel LP, Levtchenko E. Cystinosis: a review. *Orphanet J Rare Dis.* 2016;11:47.
3. Wilmer MJ, Schoeber JP, van den Heuvel LP, Levtchenko EN. Cystinosis: practical tools for diagnosis and treatment. *Pediatr Nephrol.* 2011;26(2):205-215.
4. Ariceta G, Camacho JA, Fernández-Obispo M, et al. Cystinosis in adult and adolescent patients: recommendations for the comprehensive care of cystinosis. *Nefrologia.* 2015;35(3):304-321.
5. Ariceta G, Lara E, Camacho JA, et al. Cysteamine (Cystagon®) adherence in patients with cystinosis in Spain: successful in children and a challenge in adolescents and adults. *Nephrol Dial Transplant.* 2015;30(3):475-480.
6. Langman CB, Barshop BA, Deschênes G, et al. Controversies and research agenda in nephropathic cystinosis: conclusions from a "Kidney Disease: Improving Global Outcomes" (KDIGO) controversies conference. *Kidney Int.* 2016;89(6):1192-1203.
7. Varnell CD Jr, Rich KL, Nichols M, Dahale D, Goebel JW, Pai ALH. Assessing barriers to adherence in routine clinical care for pediatric kidney transplant patients. *Pediatr Transplant.* 2017;21(7):10.1111/ptr.13027.
8. Ballantyne AO, Spilkin AM, Trauner DA. Executive function in nephropathic cystinosis. *Cogn Behav Neurol.* 2013;26(1):14-22.
9. Beinart N, Hackett RA, Graham CD, Weinman J, Ostermann M. Mood and illness experiences of adults with cystinosis. *Ren Fail.* 2015;37(5):835-839.
10. Coulter A, Entwistle VA, Eccles A, Ryan S, Shepperd S, Perera R. Personalised care planning for adults with chronic or long-term health conditions. *Cochrane Database Syst Rev.* 2015;2015(3):CD010523.
11. Badawy SM, Barrera L, Sinno MG, Kaviany S, O'Dwyer LC, Kuhns LM. Text messaging and mobile phone apps as interventions to improve adherence in adolescents with chronic health conditions: a systematic review. *JMIR Mhealth Uhealth.* 2017;5(5):e66.
12. Parker L, Ryan R, Young S, Hill S. Medications and doctor-patient communication. *Aust J Gen Pract.* 2021;50(1):709-714.
13. Raina R, Wang J, Krishnappa V. Structured transition protocol for children with cystinosis. *Front Pediatr.* 2017;5:191.



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