To nephrologists and caregivers of pediatric patients (ages 10–19) living with Alport syndrome:

Alport Syndrome Foundation's ***Milestones Tool*** is designed to facilitate important conversations that lead to knowledge, skill-building, and confidence in self-management of care over time for pediatric patients living with Alport syndrome. The tool was shaped with input from caregivers, patients, and pediatric nephrologists with expertise in diagnosing and treating our rare disease.

The tool is designed to begin use with patients at the age of 10 to help them begin to understand, accept, and become proactive about treatment for Alport syndrome based on their age and stage of disease. Reminder: Research confirms that female patients of all genetic types are not just "carriers" and are at risk of developing progressive renal disease, hearing loss, and eye conditions related to Alport syndrome.

Suggestions on how to maximize this tool:

- Download the appropriate version of the Milestones tool from our website based on the patient's age and stage of disease: https://alportsyndrome.org/about-alport-syndrome/resources-for-parents-and-caregivers-of-pediatric-patients/

- Begin with the first set of questions and make notes about the patient's responses, marking each category using the given fields of "Not Ready," "Approaching Ready," or "Ready" to transition to adult care, or to transition to the next level of questions.

- There is no expectation about how many questions will get answered at each nephrology visit or that the exact sequence of questions will be followed. The questions are designed to build conversations, relationships, and content knowledge over time.

- For skill-building with self-management, small "assignments" should be encouraged based on age and maturity, such as calling the pharmacy on their own the next time the patient only has 7–10 days of pills remaining in their prescription. This can be discussed at the next visit: "Did you do this? How did it go? Did you run into any challenges? Are you ready to take this on this responsibility each time?"

- Keep a dated copy of the tool with responses and notes in a physical or digital file to review and continue with each visit. It will be helpful for the physician and the parent/caregiver both to have copies made after each visit to assess progress.

- **For the pediatric nephrologist**: When the patient becomes 18 years of age (or whatever age limit is applied by the practice to designate a transition to adult care), it is helpful to provide recommendations for adult nephrologists as appropriate. Whether or not a new nephrologist is confirmed, it is helpful to write a letter addressed to the new nephrologist with information about the patient from your perspective and attach copies of this tool and any accompanying notes.

For pediatric patients using this tool with their caregiver and physician:

- While you may never experience end-stage kidney disease (ESKD) or need a kidney transplant, understanding and managing your symptoms, medications, and health risks associated with Alport syndrome is important, as is the transition from pediatric to adult nephrology care once you are considered an adult by the medical system.

- Your pediatric nephrologist should begin with the first set of questions and make notes about your responses, marking each category using the given fields of “Not Ready,” “Approaching Ready,” or “Ready” to transition to adult care. You will not be expected to know all the answers to these questions. The tool is designed to help make sure you learn how to manage living with Alport syndrome over time.

- There is no expectation about how many questions will get answered at each clinic visit. The questions are designed to build conversations, relationships, and content knowledge over time. It’s fine to skip around the content areas/sections of questions.

- For skill-building with self-management, small “assignments” will be encouraged based on your age and other considerations. For example, learning to communicate with the pharmacy on your own regarding refilling your medicine prescriptions. This is important preparation for when you become independent and no longer live with or are dependent on your parent/s and/or caregivers.

- Keep a dated copy of the tool with the nephrologist’s notes in a physical or digital file to review and continue with each visit. The doctor should keep a copy as well.

- If you have questions or want to share feedback on this tool with Alport Syndrome Foundation, please contact us at info@alportsyndrome.org. Our Staff and Board Members are all also affected by Alport syndrome as patients/caregivers/family members.

- This tool only addresses managing your kidney-related healthcare, but our hope and goal is that you will carry this content knowledge and skill-building over to managing additional care you may need related to living with Alport syndrome such as hearing, vision, and your emotional health.

- Note that if you would like to connect with other children, teens, or young adults living with Alport syndrome, there is a large and supportive community available to you through Alport Syndrome Foundation. All of our services, programs, projects, and virtual/in-person meetings are free of charge.

THE NEED: From the patient perspective, Alport syndrome is often particularly challenging during teen and young adult years. Young patients commonly experience disease progression while simultaneously desiring more independence from their adult caregivers. The average age of renal failure for males with X-linked Alport syndrome and males/females with autosomal Recessive Alport syndrome is 23 years old.(J Clin Med 2019 Features of autosomal recessive Alport syndrome). It is critical that pediatric patients become educated over time about living with this rare disease in preparation for making informed choices about their health, and to manage their care as a young adult living independently.

WHO WE ARE: Alport Syndrome Foundation (ASF) is a U.S.-based patient education organization with more than 8,000 free members, primarily patients and families. The organization is led by and dedicated to patients living with Alport syndrome. We provide educational resources and programs, organize events for patient connection and support, invest in research, and partner with industry to advance exploration of potential treatments or a cure for our rare genetic disease.

THE GOAL: This tool is designed to help facilitate conversation and content learning over time with patients, caregivers, and nephrologists as a team. The goal is to achieve readiness for young Alport syndrome patients to transition successfully from pediatric to adult care, and to take responsibility for their own healthcare management.

HOW THE TOOL WAS DEVELOPED: This tool was developed by ASF Staff and volunteers who have experienced this transition of care, along with guidance and input from several pediatric nephrologists and a nephrologist double certified in both adult and pediatric nephrology. With permission from Dr. Bradley Warady of Mercy Children's Hospital in Kansas City, Missouri, this tool borrows some questions and response options from a tool that his clinic uses with all pediatric CKD patients. ASF built upon and modified the tool to be focused specifically on the needs and experiences of young patients living with Alport syndrome.