

## American Society of Nephrology Kidney Health Initiative Research Priorities Survey Analysis

### BACKGROUND & RESEARCH PRIORITIES

The Kidney Health Initiative (KHI) aims to “catalyze innovation and the development of safe and effective patient-centered therapies for people with kidney diseases.” These efforts towards innovation include a Pediatric Research Priorities survey that was distributed to pediatric nephrologists and researchers.<sup>1</sup> The survey was designed to identify and understand what health care professionals see as critical priorities for people with kidney diseases. Specifically:

1. What treatment options should be explored for future use among people with kidney diseases who are 18 and under;
2. What pediatric populations should be considered for the recommended treatment option(s); and
3. Why these treatment options should be a research priority for KHI.

### CONCLUSIONS & RECOMMENDATIONS

The KHI Research Priorities Survey identified key opportunities to enhance treatment options and advance novel therapies for children with kidney diseases. While pediatric-specific data remains limited, survey participants highlight several areas that warrant focused research.

1. SGLT2 inhibitors have emerged as an effective disease-modifying treatment for CKD in the adult population. However, these therapies are not yet indicated for pediatric patients. Pediatric nephrologists and researchers express strong interest in expanding access to SGLT2 inhibitors for younger patients, hoping to see benefits similar to those seen in adults. Additional data are needed to establish safety and efficacy in the pediatric population.
  - a. Support and promote clinical trials and other data-collection efforts that will generate evidence regarding the safety and efficacy of SGLT2 inhibitors for pediatric patients. Findings may help expand clinical indications for this drug to those 18 and under with CKD, as well as rare kidney diseases such as MCD / MCNS, ANCA - GN, C3G, IC-MPGN, FSGS, Alport Syndrome, idiopathic nephrotic syndrome, Proteinuric kidney disease, steroid sensitive nephrotic syndrome, IgAN, AgAVN, and CAKUT.
  - b. Ensure that neonates and infants are adequately represented in clinical trials to avoid underrepresentation of this vulnerable subgroup.
  - c. Collaborate with researchers to explore pediatric-friendly formulations, such as liquid, slow-release, or targeted delivery options.

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<sup>1</sup> The KHI 2025 Research Priorities survey was sent to members of the American Society of Nephrology (ASN) community, the American Society of Pediatric Nephrology (ASPN) community, and the Pediatric Nephrology Research Consortium (PNRC) community.

2. Beyond SGLT2 inhibitors, respondents identify several treatments that may benefit pediatric patients with glomerular nephritis/glomerular diseases including Iptacopan/Pegcetacoplan, Obinutuzumab, and CD38 therapies.
  - a. Support and promote clinical trials and data collection to assess the safety and efficacy of these therapies among those with glomerular nephritis/glomerular diseases.
3. Respondents also indicate that hemodialysis equipment currently available is often unsuitable for the pediatric population. Equipment limitations, such as catheters that cannot accommodate neonates and infants, and a lack of access to portable or home-use devices restrict treatment options and create barriers to care, particularly for families living far from hemodialysis centers.
  - a. Identify and address barriers to availability of appropriate pediatric hemodialysis equipment, including catheters and devices specifically designed for neonates, infants, and the broader pediatric population.
  - b. Provide information and available resources to assist health care professionals and families in obtaining portable or home-use hemodialysis and peritoneal dialysis equipment.
4. The findings underscore the importance of dedicated attention to neonates and infants as a distinct subgroup within pediatric nephrology.
  - a. Establish a diverse KHI working group focused on this population's unique needs, including disease management, safety and efficacy considerations, dosing and formulation requirements, and the design of size-appropriate hemodialysis and blood pressure monitoring equipment.
  - b. A targeted approach to research and innovation for neonates and infants has the potential to significantly improve outcomes and quality of this for this population.

## SUMMARY OF FINDINGS

### Overview of Treatment Options

A total of thirty-nine (39)<sup>2</sup> individuals participated in the KHI survey. In considering treatment options that should be explored for future use in management of kidney diseases among the pediatric population, SGLT2 inhibitors is the most prevalent recommendation. Hemodialysis equipment is the next most frequently recommended treatment option.

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<sup>2</sup> Respondents from: university/school of medicine n=12, university/school of medicine + children's hospital n=9, children's hospital n=12, hospital/medical center n=2, other organization n=4.

**Table 1: Type of Drug or Device<sup>3</sup>**

Treatment	# of responses
SGLT2 inhibitors	21
Hemodialysis equipment*	11
Iptacopan/Pegcetacoplan	5
Budesonide/Nefecon	5
Obinutuzumab	5

**Total verbatim** responses for treatment options: n=102.

\***Hemodialysis equipment** includes: infant HD catheters (1), dialysis access for neonates (1), home peritoneal dialysis (1), HD filters (1), portable HD equipment (1), artificial biocompatible HD shunts (1), home machines for infants (1), smaller dialysis access options (1), all dialysis equipment (2), and smaller dialysis catheters (1).

### Overview of Target Populations

Respondents most commonly identify those with CKD and glomerular nephritis/glomerular diseases as the primary target populations for the treatment protocols identified as research priorities.

**Table 2: Target Pediatric Population for Treatment<sup>4</sup>**

Condition or age group	# of responses
CKD	28
Glomerular nephritis / glomerular diseases*	24
IgAN	9
Neonates/infants	8

**Total verbatim** responses for target pediatric population: n=142.

\***Glomerular nephritis/Glomerular diseases** include: MCD / MCNS, ANCA - GN, C3G, IC-MPGN, FSGS, Alport Syndrome, Idiopathic nephrotic syndrome, Proteinuric kidney disease, Steroid sensitive nephrotic syndrome

### Overview of Rationale for KHI Research Priority

Respondents most commonly mention treatment options seen as effective in adult populations as a rationale for exploration among pediatric populations. However, there is a note of caution as almost an equal number see a need for additional clinical data among pediatric populations.

**Table 3: Rationale for KHI Research Priority<sup>5</sup>**

Rationale	# of responses
Treatment is effective in adult population	21
Need additional data / clinical trials related to pediatric population	19
Need expanded / alternative treatment options	16
Need devices appropriate for pediatric population	14

**Total verbatim** responses for rationale for research priority: n=154

<sup>3</sup> Table 1 only includes treatments with five (5) or more responses.

<sup>4</sup> Table 2 only includes top four (4) target populations responses.

<sup>5</sup> Table 3 only includes top four (4) rationale for priority responses.

## **Making the Case: Connecting Treatment Recommendations to Target Populations**

Evaluation of research priorities involves a review of the treatment options aligned with the populations to be considered. Health care professionals identify the most likely target populations for the top recommended treatment options:

**SGLT2 Inhibitors:** (n=21)<sup>6</sup> A majority who identified SGLT2 inhibitors as a research priority see CKD and glomerular nephritis/glomerular diseases as primary target populations. Several also see those with CAKUT as a population that could benefit.

**Hemodialysis Equipment:** (n=11) Those on dialysis and/or with ESKD/ESRD are seen as target populations.

**Iptacopan/Pegcetacoplan:** (n=5) Those with IgAN or glomerular nephritis/glomerular diseases are seen as target populations.

**Budesonide/Nefecon:** (n=5) Those with IgAN or IgAVN are seen as target populations.

**Obinutuzumab:** (n=5) Those with glomerular nephritis/glomerular diseases are seen as target populations.

## **Making the Case: Connecting Treatment Recommendations to Research Priority**

Health care professionals provide rationale for the top recommended treatment options.

**SGLT2 Inhibitors:** (n=21)<sup>7</sup> Treatment is effective in adult population, need additional data / clinical trials related to pediatric population, need expanded / alternative treatment options, to better inform IgAN management/diseases management, better understand safety and efficacy of adult therapies, need therapies to slow progression of CKD, improve insurance process, and improve metrics and monitoring

**Hemodialysis Equipment:** (n=11) Need devices appropriate for pediatric population, need improvements in access to dialysis

**Iptacopan/Pegcetacoplan:** (n=5) Need additional data / clinical trials related to pediatric population and a different formulation of drug may be beneficial

**Budesonide/Nefecon:** (n=5) Treatment is effective in adult population, better understand safety and efficacy of adult therapies, need additional data / clinical trials related to pediatric population, side effects of currently available drugs, and to better inform IgAN management/diseases management

**Obinutuzumab:** (n=5) need additional data / clinical trials related to pediatric population and need expanded / alternative treatment options

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<sup>6</sup> Target populations with two (2) or more mentions for treatment option, listed in order of frequency.

<sup>7</sup> Research priority rationale with two (2) or more mentions for treatment option, listed in order of frequency.