Riley Hospital for Children
Indiana University Health

SCHOOL OF MEDICINE
DEPARTMENT OF PEDIATRICS

March 17, 2025

Representative Timothy O'Brien

Re: House Bill 1453

Dear Representative O'Brien,

The Rare Disease Advisory Council appreciates the consideration of House Bill 1453. We would like to provide the following evidence in support of this bill. Briefly, a survey was conducted by the Rare Disease Advisory Council with approximately 80% of all Indiana clinical pediatric oncologists responding. The survey covered a wide range of topics, including:

- Access to clinicians/services
- Access to testing and medications
- Access to information and support services
- Variation in getting needed services
- Travel for services
- Research and research access
- Biggest problems in providing care and its
    - Impact on quality of care
    - Impact on outcomes
- Opportunities and challenges

A need for funding was a theme. This includes funding for basic research as well as clinical trials and health services research to measure long term clinical outcomes. Funding was reported to attract pediatric-specific basic science and translational researchers and availability of seed funding to start projects while trying to get larger grants or other types of financial support. There is also a lack of funding for new Children's Oncology Group protocols and National Cancer Institute-sponsored clinical research. We also need to test the establishment of statewide networking and partnerships to expand mental health and benefits of home health services. Finally, there is a need to study the socio-economic gap in receiving the best care, i.e., those with financial resources have access to the best care while those with limited financial and social resources may not as easily access or demand the best care.

The following summarizes detailed responses related to research – it is important to note that this survey included physician scientists and clinicians.

> Eighty-seven percent of respondents reported being involved in clinical trials, 53% in other clinical research, and 6.7% in bench (basic science) research (numbers add to

more than 100% because respondents could be involved in multiple types of research).

Twenty percent of clinicians stated that it was not a problem (not difficult) for their patients to participate in new research studies. While 67 percent said it was a slight problem. Of note, 14% either didn't answer the question or said they did not know.

Respondents were asked where families go to participate in research studies: 100% reported at their own institution; twenty percent reported another institution within Indiana, and 53% reported an out-of-state institution. (numbers add to more than 100% because respondents can have some patients who participate in different studies in different places).

Sixty percent of clinicians reported having at least five patients participate in a clinical trial network study within the past three years. Reasons patients have not participated included patients not meeting study criteria, no open study for the patients they see, and difficulty finding study criteria.

Ninety-three percent of clinicians (with at least five patients participating in a clinical trial network study) participated in the Children's Oncology Group. Others include BMT CTN (13.%), PBTCTC (13%), PNOC (20%), CureWorks (7%), PTCTC (7%) and other pharma studies (7%).

Respondents reported that families are educated on the availability of research studies at the time of diagnosis and during the course of treatment when possibly eligible by the clinician or the research team.

The most common reasons clinicians reported for patients choosing NOT to participate in recommended clinical trials were concern about the risks/side effects and not being interested in research, followed by distrust of research, requirement for extra tests or procedures, and time constraints.

Opportunities and Challenges

There were *two questions* about opportunities and challenges:

The first question was: *Now, thinking about pediatric cancer care in all of Indiana, what do you see as the* ***greatest possibility*** *for developing new or improved childhood cancer therapies using the talent and resources available in Indiana?*

Two themes emerged from the responses. The first was regarding current research work and strengths. Riley has a large number of clinical faculty and strong basic scientists, which could lead to further bench-to-bedside collaboration. For example, studies coming out of the research labs inform how to utilize existing drugs to treat new neoplasms in pediatric and young adult patients. There are a growing number of clinical trials available at Riley, including CART-T Cell therapy. Opportunities reported regarding clinical research were centered around increased pooling of resources and collaboration, further ensuring that clinical talent is connected to resources and made aware of what is available.

The second opportunity for developing new or improved childhood cancer therapies was related to health services research. The first theme was the need to explore and test better means of providing mental/behavioral health care that would lead to better short and long-term outcomes. The second theme was the need to discover and test better ways of providing a transition to adult care for cancer patients – many of whom are not aware of the potential late effects of cancer therapies.

The second question was: *What do you see as the* ***greatest challenge*** *for developing new or improved childhood cancer therapies using the talent and resources available in Indiana?*

Themes mirrored the opportunities question, but also several people felt that we need to think further, i.e., not just to be the best in Indiana but to be a national model for innovation, improved translation of bench to bedside to community care, more collaborative care, and more comprehensive, holistic care.

New potential therapies are coming out of the research labs, but getting those new therapies into patients can be a daunting task. Respondents reported a lack of time, resources, and investments. There is a need for support staff to support these trials and more protected time for physicians to lead them. One physician stated, “There are many of us who are interested in pursuing research opportunities, but the support system is not there to step away from the bedside. We need additional resources to carry out the care on the clinical side for us to be effective. You do not need to recruit 100% researchers, but you do need to protect the time of those who want to do research.” Another said, “If we want new therapies, we need Advanced Practice Providers to help cover patients to allow for time to develop concepts.” A third reported, “[We] need protected time for faculty… to be able to create and lead translational and clinical research studies, which requires creating more non-physician support systems for help with administrative and other tasks that don’t require a physician to do them. Clinical research teams are currently swamped with opening studies from the major consortia so that new research will require more clinical research associates.

Finally, a respondent reported concern about the decline in the number of medical students and residents choosing pediatric hematology-oncology as a career. Published research reports a shortage of pediatric oncologists and that trainees are choosing careers with better work/life balance and better pay.

Respectfully submitted,

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