CRITICAL PATH INSTITUTE 20 YEARS OF IMPACT

Advancing Drug Development. Improving Lives. Together.

March 2025

**Dear BmDR Enthusiasts,**

This newsletter serves to inform and connect everyone involved in contributing to and analyzing data in the Biomarker Data Repository (BmDR). Your participation is essential to advancing this effort. Collaboration remains a critical factor in ensuring the success of this initiative. We encourage you to engage with us and contribute to this collective effort. This edition specifically reviews the strategy and vision of the BmDR, including the 2025 goals, a review of the data currently in the repository, and details about related upcoming events.

Sincerely,

***Nicholas King***

Executive Director
Predictive Safety Testing Consortium

***Katrina Peron***

Associate Director
Predictive Safety Testing Consortium

## What is the Biomarker Data Repository (BmDR)?

BmDR is a public repository for novel translational safety biomarker data from a variety of independent academic and corporate sources. The repository provides users with a large, reliable dataset on novel translational safety biomarkers from a variety of nonclinical and clinical study sources.

Masked, de-identified data from multiple sponsors are being collected and stored in a secured repository. The data will be available to C-Path and BmDR members, and requestable by qualified researchers, to support research that leads to the submission of documents to global regulatory agencies to qualify novel safety biomarkers for new Contexts of Use (CoUs), to modify and expand existing CoUs and to identify appropriate exploratory safety biomarkers to advance drug development in the future.

For additional questions on BmDR, view both the Technical and Patient-Focused FAQs.

### Video Resource: BmDR DAP Overview February 2025.

## 2025 BmDR Goals

1. Complete Data Contribution Agreements with those organizations currently engaged (>10 clinical datasets)
2. Draft data analysis plan with patient level thresholds for data in the data analytics platform
3. Prioritize data sets to pursue to add to the platform
4. Develop three-year strategies for the BmDR Communications & Publications and Community Engagement Committees

## BmDR Vision

**Near-Term**
Through continual importation of standard and novel biomarker data from academic researchers, as well as pharma Early Clinical Development (ECD) and non-ECD studies under C-Path's BmDR Data Contribution Agreements, it is anticipated that qualified novel biomarker data and non-qualified biomarker data will provide opportunities to understand the performance of the biomarkers across (i) the full age spectrum, (ii) baseline kidney disease improving global outcomes of chronic kidney disease glomerular filtration rate strata, and (iii) a broad spectrum of common and uncommon kidney diseases. Evidentiary data standards will be based upon biomarker assays quality and deidentified patient-level data (e.g., demography, medical history, biometrics [e.g. height, weight, BMI], and other relevant factors). Data standards and quality will be maintained by the C-Path BmDR Data Analytics Platform in accordance with Clinical Data Interchange Standards Consortium, Study Data Tabulation Model, and Fast Healthcare Interoperability Resources standards.

**Long-Term**
The C-Path BmDR DAP will facilitate qualified researchers' access to high quality deidentified patient-level data, modeling expertise, artificial intelligence/machine learning capabilities, and relevant publications (BmDR-based and peer-reviewed journals-based) to contextualize and potentially expand the utility of validated tissue and body fluid biomarkers, digital histologic imaging, digital radiographic imaging, as well as incorporate non-validated novel biomarkers. As a nexus of high-quality tissue, biofluid, and digital imaging patient-level data, the BmDR DAP wealth renal injury (tubular and/or glomerular) biomarker data, renal function (estimated and measured) data, and renal histologic and renal parenchymal imaging can facilitate knowledge transfer and knowledge expansion through robust analyses and expand regulatory acceptance of multifunctional safety and efficacy assessments beyond current capabilities.

## Latest Developments

The Predictive Safety Testing Consortium's Kidney Safety Project (KSP) team received a positive determination letter from the FDA on December 6, 2024, for the Qualification Plan (QP) for a Novel Biomarker Panel (NBMP) to be used in conjunction with standard laboratory tests to indicate response to drug-induced injury to the kidneys. This would support early detection, monitoring, and potential reversibility of kidney injuries during clinical trials. With QP acceptance, the group has initiated the statistical analysis plan and begun drafting the Full Qualification Package to be submitted to the FDA requesting qualification of the biomarker panel. Read the press release here.

C-Path and Boston Medical Center (BMC) published results for six biomarkers that could improve the early and accurate detection of kidney injury, leading to both the development of safer medications and better health outcomes for all patients. The results leverage the collaborative expertise and investment of the C-Path Predictive Safety Testing Consortium's (PSTC) Nephrotoxicity Working Group, and the Clinical Evaluation and

Qualification of Translational Kidney Safety Biomarkers Project, a public-private partnership managed collaboratively by the Foundation for the National Institutes of Health (FNIH) Biomarkers Consortium Kidney Safety Biomarker Project Team. The findings from this collaboration were published in Clinical Pharmacology & Therapeutics. These biomarkers may offer an approach to detect drug-induced kidney damage earlier than existing standards for monitoring kidney health, and can lead to more tolerable treatment options.

The KSP team has been working closely with the BmDR Oversight Committee Co-Chairs on the impact of their work to patients and the community. Patients with kidney disease have goals which include, (but are not limited to):

- Avoiding or delaying the need for dialysis and/or transplantation.
- Maintaining good health to enjoy family life and good employment to ensure economic stability.
- Finding greater medical predictability from nephrologists with awareness of emerging tests and treatments.

The PSTC NBMP has the potential to achieve these goals:

1. If a patient develops new symptoms between planned visits to nephrologist [when standard labs are done (serum creatinine, eGFR, cystatin C, urinalysis)], the NBMP may provide confidence that kidney function is not at risk OR that kidney injury requires prompt addressing with their nephrologist.

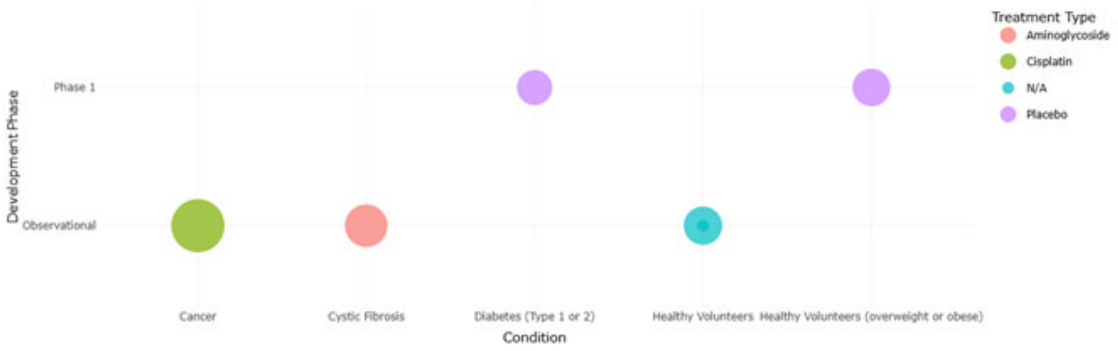
2. Potentially nephrotoxic drugs do not cause injury to all patients. A patient receiving and benefitting from this type of medicine could have novel biomarkers measured to determine if any sign of Acute Kidney Injury (AKI) is detected.

3. Patients with kidney disease, who have this information generated, can altruistically advance the science to benefit others with kidney disease.

If you are interested in participating in the BmDR Oversight Committee or one of the other committees, please contact Nick King or Katrina Peron.

## Data in the BmDR

Data currently in the BmDR includes studies that have assessed drug induced kidney injury (DIKI), including with healthy volunteers, people living with type 1 and type 2 diabetes, subjects with cystic fibrosis, and cancer patients. These conditions may contribute to DIKI in different development phases. BmDR also includes non-clinical (rat) data containing biomarkers of interest. The breadth of current BmDR data — how many patients and from what type of studies — is shown pictorially and in the table below. These studies have helped show what types of treatments may be nephrotoxicants, or induce injury to the kidney. This also helps show what other disease data may be useful to the BmDR, such as hypertension or renal disease. By combining other studies assessing biomarkers with what's already in the BmDR, we can create a much clearer picture of these biomarkers for the community.




| Condition | Clinical Stage | | Nonclinical (Rat) | Grand Total Subjects |
|---|---|---|---|---|
| | Phase 1 | Observational | Nonclinical | |
| Cancer | - | 140 | - | 140 |
| Cystic Fibrosis | - | 95 | - | 95 |
| Diabetes (Type 1 or 2) | 72 | - | - | 72 |
| Healthy Volunteers | - | 121 | - | 121 |
| Healthy Volunteers (overweight or obese) | 79 | - | - | 79 |
| Not Applicable (Nonclinical) | - | - | 160 | 160 |
| **Grand Total Subjects** | **151** | **356** | **160** | **667** |

## Accessing Data on BmDR

Qualified researchers can request access to the BmDR DAP to review and interrogate data, as well as request specific datasets, using this link: https://fair.dap.c-path.org/#/data/collections/bmdr-data-collection. For further instructions and details on how to review and request datasets on the DAP, watch this video.

## Contributing to BmDR

The BmDR team is in talks with organizations for an additional ten clinical datasets, which will aid in our goal of expanding the volume and capabilities of the repository. Outreach continues to consortia, academic researchers, and companies with relevant data sets to contribute.

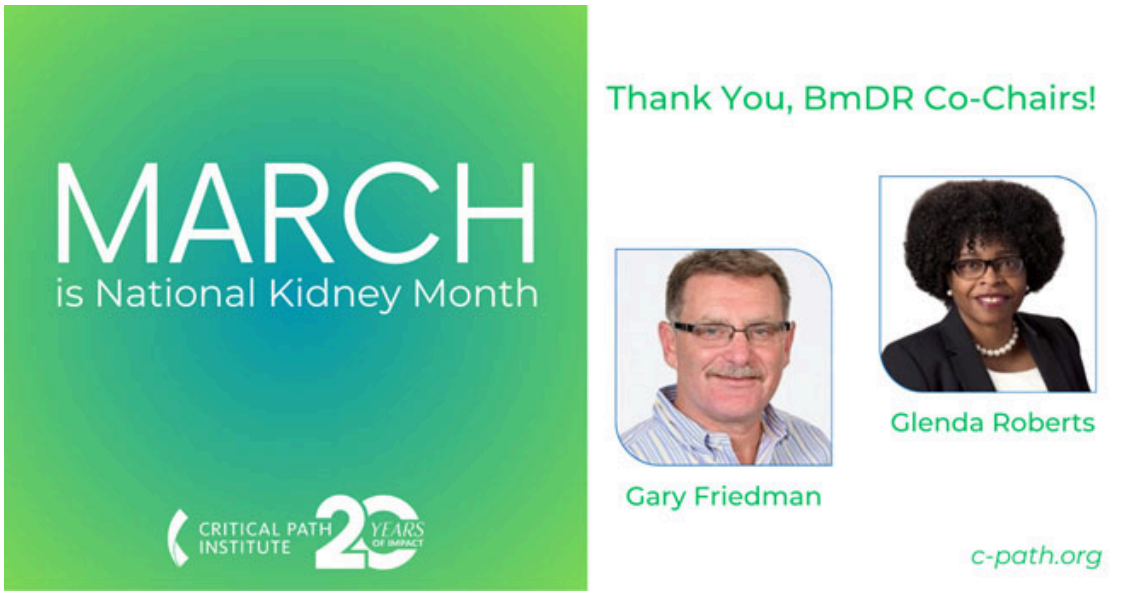
**Please consider contributing your data to BmDR. Populations of interest to the team include pediatric study populations. If interested, to discuss further, please contact bmdr@c-path.org, or Nicholas King.**

## March is World Kidney Month!

World Kidney Day (March 13) aims to raise awareness of the importance of our kidneys to our overall health and to reduce the frequency and impact of kidney disease and its associated health problems worldwide. World Kidney Day is a global campaign aimed at raising awareness of the importance of our kidneys. Make sure to follow along C-Path's social media highlighting our efforts during World Kidney Month.

World Kidney Day is a joint initiative of the International Society of Nephrology and the International Federation of Kidney Foundations – World Kidney Alliance.

We would especially like to thank BmDR co-chairs Gary Friedman and Glenda Roberts for all their help with BmDR!




## Save the Date for CGIC 2025!

For 20 years, Critical Path Institute ® (C-Path) has been a trusted nonprofit that generates solutions to facilitate scientific and regulatory pathways that accelerate the development of therapies for people with unmet medical needs. Join the BmDR team at the 2025 C-Path Global Impact Conference (CGIC), September 9 – 11, at the Washington Marriott at Metro Center, D.C. CGIC is a unique opportunity to connect the dots across key initiatives within C-Path's valuable portfolio, dedicated to accelerating drug development for conditions in neurology, rare diseases, pediatrics, and more. Registration for the event will be open soon.

We hope to see you there!

## Recent Publications:

"Urinary Kidney Injury Biomarker Profiles in Healthy Individuals and After Nephrotoxic and Ischemic Injury." Waikar, Sushrut S., Robin Mogg, Amanda F. Baker, Gyorgy Frendl, Michael Topper, Scott Adler, Nicholas MP King, Stefan Sultana, et al. 2025. Clinical Pharmacology & Therapeutics.

"Serum Glutamate Dehydrogenase Activity Enables Sensitive and Specific Diagnosis of Hepatocellular Injury in Humans." Aubrecht, Jiri, David Potter, John Michael Sauer, Roscoe Warner, Kent Johnson, Mitchell R McGill, Katrina Peron, and Nicholas M P King. Toxicological Sciences, November 6, 2024.

"Leveraging Biomarkers and Translational Medicine for Preclinical Safety - Lessons for Advancing the Validation of Alternatives to Animal Testing." Hartung, Thomas, Nicholas M. P. King, Nicole Kleinstreuer, Marcel Leist, and Danilo A. Tagle. ALTEX - Alternatives to Animal Experimentation 41, no. 4 (October 22, 2024): 545–66.

## At a Glance

BmDR Website
Past BmDR Newsletters and Resources
BmDR Data and Analytics Platform (DAP)

If you have any questions about BmDR, please email bmdr@c-path.org.

Help support our mission. MAKE A GIFT TODAY

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