PCORnet’s Cancer Moonshot Commitment
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On Friday, May 27, 2016, the leaders of PCORnet, the National Patient-Centered Clinical Research Network, submitted the following application to the White House Cancer Moonshot Website outlining the network’s commitment to accelerate progress in cancer research and care.

Tell us about the commitment you're making.

PCORnet, the nation’s largest and most innovative patient-centered clinical research network, strongly supports the Vice President’s Cancer Moonshot and would be delighted to work with the White House Cancer Moonshot Task Force, federal agencies, and private sector partners to help advance the Moonshot’s mission. PCORnet is an initiative conceptualized and funded by the Patient-Centered Outcomes Research Institute (PCORI) as a means of supporting efficient, patient-centered, “real-world” clinical research to improve health. PCORI is an independent, non-profit organization authorized by Congress in 2010. Its mission is to fund research comparing which healthcare options will work best for patients given their particular concerns, circumstances, and the outcomes most important to them.

Specifically, PCORnet is committed to:

1. Enhancing our highly representative national clinical research network—one that includes data from more than 110 million patients who received their care over the last 5 years, supports a learning U.S. healthcare system, and enables large-scale research, including cancer research, to be conducted with enhanced quality and efficiency.

2. Creating data tables that include descriptive characteristics of patient cohorts treated for the most common types of cancer (including breast, colorectal, prostate, lung, and melanoma) within PCORnet’s 130 healthcare systems. With additional funding, we could commit to enhancing these tables with descriptions of treatments and outcomes by incorporating tumor characteristics, chemotherapy regimens, radiation treatments, and more.

3. Planning a cancer collaborative research group (CRG) affiliated with PCORnet that brings together teams of patients, clinicians, and investigators to 1) identify and prioritize our nation’s top research questions; 2) develop approaches for using real-world electronic data to address unmet research needs in prevention, diagnosis, and treatment; and 3) reduce disparities in cancer care and outcomes.

With additional collaboration from the Cancer Moonshot and/or other funding partners, we can also commit to:
4. Conducting new patient-driven cancer research, including outcomes research, by engaging patients with colorectal or breast cancer whose data are currently contained in large cohorts within PCORnet’s Clinical Data Research Networks, including those derived from large, pragmatic clinical trials.

5. Working with other sponsors of real-world clinical data collection and analysis related to cancer diagnosis, treatment, and surveillance, including the American Society of Clinical Oncology’s (ASCO) CancerLinQ Program and others with similar missions. The aims of such collaborations are to harmonize cancer-related data from diverse electronic health record systems for use in large, collaborative research and quality improvement programs.

Tell us more.

Enhancing our highly representative national clinical research network—one that includes data from more than 110 million patients who received their care over the last 5 years, supports a learning U.S. healthcare system and enables large-scale research, including cancer research, to be conducted with enhanced quality and efficiency.

PCORnet’s “network of networks” encompasses rich data drawn from 110 million patients in diverse settings across the country and offers cancer researchers a potential wealth of real-world insights. It has been built through close and ongoing consultation and collaboration with multiple federal agencies, including the NIH, FDA, ONC, ASPE, CDC, and AHRQ as well as with the life sciences industry – the future users of PCORnet. Most importantly, patients and their advocates have played an active and central role in PCORnet’s development and governance from its inception.

To support the Vice President’s Cancer Moonshot, we are committing to growing and enriching our data. We are also committed to continuing to enhance our robust infrastructure, which unites patients, clinicians, and health systems in efforts to leverage the enormous volumes of data generated through routine interactions with the healthcare system. To accomplish this goal, we are linking millions of previously disparate data points, such as information from electronic health records (EHRs), patient-reported outcomes, and other information contributed directly by patients and healthcare consumers, and inviting clinicians, health systems, and patients to be active participants in the research process from the outset. Connecting these data will allow us to follow patients throughout the healthcare system, starting before their diagnosis, through treatment and on to longer-term outcomes. Importantly, we will be able to integrate information from all of their healthcare providers—not just the specialists managing their cancer.

PCORnet is made up of two kinds of complementary partner networks—Clinical Data Research Networks (CDRNs) and Patient-Powered Research Networks (PPRNs). Our 20 PPRNs are operated and governed by patient groups and their partners who are motivated to play an active role in patient-centered clinical research. Our 13 CDRNs are networks that originate in
healthcare systems, such as hospitals, integrated delivery systems, academic medical centers, or practice-based networks. They securely collect health information during routine patient care. These 13 CDRNs encompass approximately 130 different health systems and settings from across the country.

Examples of the deep experience relevant to the Cancer Moonshot that our CDRNs and PPRNs offer include:

- **The PORTAL CDRN** (Patient Outcomes Research to Advance Learning) colorectal cancer cohort, which currently holds detailed clinical data on more than 16,000 patients from six integrated health systems, who were diagnosed between 2010 and 2014. PORTAL maps data from different cancer registries, EHRs, and other data sources into a common data model to facilitate research across multiple sites. PORTAL sites are part of the NCI-funded Cancer Research Network. PORTAL has also collaborated with Smart Patients in creating online communities of patients who can provide input on a variety of topics.

- **The GPC (Greater Plains Collaborative) breast cancer CDRN**, which includes eight NCI-designated cancer centers affiliated with 13 institutions. A data repository containing oncology registry and EHR data for consenting subjects has been established; it contains variables identified as high priority for the research goals of the breast cancer group.

- **The PEDSnet Pediatric Learning Health System CDRN**, a large national community of hospitals and healthcare organizations, researchers, clinicians, patients and families that encompasses eight pediatric health systems. This community works together to identify the most important research questions that can reduce children’s suffering and support their healthy development. PEDSnet represents a rich source of data drawn from one of the nation’s largest experiences of pediatric cancer treatment.

- **The ABOUT PPRN** (American BRCA Outcomes and Utilization of Testing Patient Powered Research Network), which is the result of a 10-year collaboration between the leading national nonprofit advocacy organization for individuals and families impacted by hereditary breast and ovarian cancer and a team of researchers at the University of South Florida.

PCORnet investigators have already built observational data sets on large cohorts of patients with colorectal cancer, breast cancer, and hereditary ovarian and breast cancer, as well as pediatric patients with childhood cancer. The vast scale of PCORnet also supports research in rare cancers.

To maintain data security, we have built PCORnet as a distributed research network in which all the data used for studies remains behind the firewalls at PCORnet’s partner networks. For more information, please visit [www.pcornet.org](http://www.pcornet.org).

**Describing the characteristics of patient cohorts treated at one of PCORnet’s 130 participating health systems for important types of cancer (including breast, colorectal, prostate, lung and melanoma) in the past 5 years. With additional funding, we could commit to enhancing these data tables to include detailed information about cancer stage and initial treatment from**
cancer registries, real-world experiences with specific chemotherapy protocols and radiation treatment, and long-term utilization and comorbid conditions.

We are committed to building detailed, accessible, aggregate data about patients receiving care from PCORnet-affiliated systems that would provide the foundation for identifying and addressing major questions in cancer treatment as well as for designing studies. These tables would include descriptive characteristics of curated cohorts of patients diagnosed with leading types of cancer, including breast (~143,000), colorectal (~40,000), prostate (~119,000), lung (~58,000), and melanoma (~26,000). These data tables will include patient demographic characteristics and other features, such as other diagnoses, procedures, results from laboratory and other tests, and treatments. These data can be used for a wide array of purposes, such as constructing patient profiles, describing the use of different treatment approaches, and identifying disparities in care.

Our PORTAL colorectal cancer cohort shows what we can accomplish with our data. PORTAL currently includes 16,212 patients, 49% of whom are female and 51% male. About 10% were diagnosed before age 50; approximately 20% were diagnosed at age 80 or older. The cohort is 63% white, 10% African American, 11% Asian/Pacific Islander, and 15% Hispanic, and includes a good distribution across stage of diagnosis. A random sample of 4,000 of these patients were surveyed to learn about their experiences with treatment, what they wished they had known before they had to make treatment decisions, their willingness to participate in research, and other similar questions that were developed in collaboration with patients. Because these patients receive treatment at integrated health systems, we can observe their patterns of care before they were diagnosed and follow their outcomes for many years after they complete treatment.

Another illustrative example is the GPC breast cancer cohort. It initially included 4,578 women with breast cancer diagnosed during 2013 and 2014 with merged tumor registry and EHR data. These data were linked with patient-reported outcomes collected with a 20-page mailed questionnaire designed in partnership with patients. Of 1,986 patients surveyed, 1,235 responded (62%) and medical record consent was granted by 69% of respondents. The mean age at diagnosis was 59 and mean FACT-B quality of life score was 115. The cohort is 93% white, 4% African American, 3% other race, and 4% Hispanic. Hormone receptor status was 83% estrogen receptor positive, 73% progesterone receptor positive, and 13% HER2 positive. Stage distribution in the surveyed cohort includes 16% stage 0, 46% stage I, 28% stage II, and 10% stage III. Positive BRCA1 test results were reported by 3% of women and bilateral mastectomy was reported by 23%. The cohort continues to expand as new patients are diagnosed and new health systems are added.

With additional funding, we could enhance these curated cohorts to include registry-linked tumor characteristics, chemotherapy regimens, claims data, genomic data, biospecimens, and radiation treatment data. While the basic table is already in development within PCORnet, we estimate that the enhanced table could be completed in approximately 12 months.
Planning a cancer collaborative research group (CRG) affiliated with PCORnet that brings teams of patients, clinicians, and investigators together to identify our nation’s top cancer research questions and approaches for using PCORnet to address unmet medical needs.

We are planning to build a national cancer CRG with the capacity to use the diverse, real-world data provided by PCORnet and cancer registries to support both large observational outcomes studies and pragmatic clinical trials in cancer prevention, diagnosis, and treatment. The cancer CRG will harness the combined power of patients, clinicians, cancer researchers, and health system leaders associated with multiple CDRNs and PPRNs within PCORnet to identify the nation’s top cancer research questions and develop approaches that can fully leverage the enormous potential represented by PCORnet’s EHR data and infrastructure. The cancer CRG would be the facilitative front door for connecting Moonshot ideas (rockets) with the means for getting the work done (landing zones).

Conducting studies through the cancer CRG will require additional funding for PCORnet from PCORI, the National Cancer Institute, the life sciences industry and/or funding identified through the Moonshot program. We will apply PCORnet resources to this work, including investigators and availability for network development of data assets. Specifically, PCORnet’s CRG will:

- Identify interested cancer researchers across the 130 healthcare delivery systems within PCORnet.
- Create, validate, share and expand cancer-related data elements within PCORnet by leveraging what the NCI-funded Cancer Research Network has developed.
- Collaborate with the FDA’s Sentinel Initiative to develop the capacity to conduct standardized queries on our rich data set.
- Collaborate with cancer registries in PCORnet sites (including internal registries, those that report to SEER, and CDC State Cancer Registries) to enhance the completeness of cancer diagnosis, treatment, and outcome data.

With additional collaboration from the Cancer Moonshot and/or other funding partners, we can commit to conducting new patient-driven cancer research by engaging patients whose data are currently contained in large cohorts of colorectal or breast cancer within PCORnet’s Clinical Data Research Networks.

PCORnet is committed to changing the research conversation from one directed largely by researchers to one driven by the needs of patients and other healthcare stakeholders. We are currently pioneering studies that feature an unprecedented degree of patient guidance; we believe these efforts provide a model for how future studies should be conducted.

For example, our ADAPTABLE aspirin study is a 3-year pragmatic clinical trial comparing the effectiveness of two different daily doses of aspirin, both of which widely used to prevent heart attacks and strokes in persons living with heart disease. Patients with heart disease helped design the ADAPTABLE protocol, consent form, patient web portal content, and study materials.
They are an integral part of the research team and serve as members of the executive and steering committees. PCORnet is using this approach to shift the role of patients from that of “study participant” to a “research partner” who helps determine what to study and how.

Another example is the PORTAL project (described previously), which has collaborated with Smart Patients (https://www.smartpatients.com/home) to create virtual communities of colorectal cancer patients and caregivers. Smart Patients has developed approaches to engaging patients in designing clinical trials that work for patients by using the principles of user-centered design. With additional funding, we will experiment with applying these approaches to multiple clinical trials in order to develop evidence about elements of patient-centered clinical trial design that are shown to improve recruitment and retention.

In addition, our ABOUT PPRN (American BRCA Outcomes and Utilization of Testing Patient Powered Research Network) is the result of a 10-year collaboration between the leading national nonprofit advocacy organization for individuals and families impacted by hereditary breast and ovarian cancer and a team of researchers at the University of South Florida. These partners—led by patient advocates—have combined their strengths in advocacy, research, and community engagement to pursue better information, services, and outcomes for the patient community to which they belong, and to conduct collaborative research that involves patients at every level.

Finally, the PORTAL colorectal cancer group has partnered with Fight Colorectal Cancer (http://fightcolorectalcancer.org/) to identify research questions important to colorectal cancer patients and their families. One area of interest centers on survivorship—in other words, what patients who have survived colorectal cancer can expect after treatment, including the health and lifestyle effects of treatment. The group has also identified an interest in young colorectal cancer patients whose cancer is diagnosed at an age before traditional screening begins.

With additional funding, we can commit to leveraging these strategies and more to conduct new patient-centric research that answers the questions that matter most to cancer patients.

With additional collaboration from the Cancer Moonshot and/or other funding partners, we can commit to working with other sponsors of real-world clinical data collection on cancer diagnosis, treatment and surveillance, including ASCO’s CancerLinQ Program and others with similar missions. The aims of such collaborations are to standardize the extraction and curation of cancer-related data from diverse EHR systems for use in large collaborative research and quality improvement programs.

Several efforts spanning public and private sectors are currently underway within the United States to capture clinical data on tumor characteristics and treatments from EHRs. Among these are PCORnet, ASCO’s CancerLinQ, and Flatiron’s performance improvement databases. Because each draws data from a variety of EHRs that are not standardized, they must extract and transform the data so that it can be aggregated and used for research and/or quality improvement. There is a critical need for work to harmonize these and other approaches to
data standardization in cancer research and surveillance so that the large studies needed to understand subgroup differences in treatment effectiveness can be accomplished, even for relatively rare tumors. Each of these efforts has unique features and the patient populations in all three efforts will overlap greatly. Thus, there are many reasons why the capacity to standardize and link data will be valuable for pragmatic, real-world studies of cancer treatment outcomes, as well as studies of cancer surveillance following treatments. PCORnet has confirmed with ASCO that there is strong mutual interest in pursuing this activity. We are currently reaching out to Flatiron to confirm their interest in collaborating as well.

Additional funding would also support the ability for us to link existing cancer registries with PCORnet and integrate them with our Common Data Model. This would allow for valuable insights into the management of cancer within the larger context of care delivery.

Anything else?

There are numerous ways to leverage PCORnet to support the Cancer Moonshot. The following ideas illustrate the ways in which PCORnet could advance the pace of cancer research:

- Use PCORnet to survey thousands of cancer patients to obtain patient-reported experiences with cancer treatments, including in underserved communities (short term: 3-6 months).
- Use PCORnet to advance the use of patient-reported outcomes in clinical trials (medium to long term: 6-12 months).
- Use PCORnet to evaluate adoption of cancer drugs in clinical settings, evaluate the real-world effectiveness of alternative therapies, and collect survey data (including patient-reported outcomes) from patients (medium term: 6-9 months).
- Use PCORnet to improve shared decision-making so that patients receive preferred treatments based on their individual risks, preferences, and values (long term: 12+ months).
- Use PCORnet to test approaches to implementing at scale successful approaches to cancer prevention and early detection (medium to long term: 6-12 months).
- Use PCORnet to launch large pragmatic clinical trials comparing available therapies as they are used in real-world settings and engage patients in the design of those trials (long term: 12+ months).
- Explore the use of molecular testing to improve our understanding of heterogeneity in current cancer diagnosis classification systems (long term: 12+ months).
- Explore mechanisms for identifying subgroups of patients most likely to benefit from specific types of cancer treatment and for bringing that information into care delivery (long term: 12+ months).
- Engage with the currently forming Health Systems CRG to implement specific approaches to reducing or eliminating disparities in cancer prevention, screening, and treatment (long term: 12+ months).
- Use PCORnet for surveillance of diffusion of new therapies, including early observational investigation of short-term outcomes and safety events.
Use PCORnet to glean insights that help us better understand the patient experience with cancer treatment. PORTAL included this in its cohort survey and received useful feedback on what information people wished they had known before they started treatment. This also lends itself to developing information that can facilitate doctor-patient decision-making.

Engage closely with patient communities to identify ways in which cancer treatment could be improved and then work on implementation.

Identify comparative effectiveness of a variety of cancer treatments, including side effect profiles, for different population subgroups. For example, PORTAL has previously used data collected through the Beacon module in the Epic EHR system to study the safety of different chemotherapy/infusion regimens for patients with advanced stage cancer.

Explore new insights about cancers from biomarker work, molecular testing, and more. For example, PORTAL is currently conducting a pilot study on characterizing colorectal cancers based on molecular markers; however, it is the combination of the laboratory and pathology information with other data on patients, clinical characteristics, and environments that is most powerful and compelling.

Facilitate patient engagement in the design and recruitment of clinical trials. For example, PORTAL has worked with Smart Patients, which has used online communities of cancer patients to co-design clinical trials.