

DD12-1206 Final Performance Report
California PHRESH Project
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1. *Provide a 2-3 sentence summary statement of the progress, or lack of progress, toward the achievement of the originally stated aims:*

- A validated case definition and methodologies for collection of surveillance data on persons with sickle cell disease/thalassemia
 - We have completed a thorough review of the methodologies that were used to conduct surveillance for sickle cell disease (SCD) and thalassemia during the RuSH project, and have compiled an extensive report and specific recommendations for future surveillance efforts for these disorders.
- Baseline estimates of the demographics and health service utilization of persons with sickle cell disease/thalassemia, especially in the areas of vaccinations, screening for complications, and disease-modifying therapies
 - We developed these estimates early in the project for SCD, and disseminated in the form of fact sheets for professional and lay audiences. These are available from a variety of sources and have been distributed to thousands of pediatric and primary care providers who are likely to see SCD cases. We estimated race and ethnicity specific incidence rates for major subtypes of thalassemia and disseminated in the form of fact sheets to physicians in areas populated with high risk populations. Estimates on health service utilization were more difficult to develop for thalassemia but we made recommendations for how to better accomplish this in future efforts (see Appendix 1: Validation Report for specific details).
- Development, dissemination, and evaluation of key messages for population-targeted health education materials, including persons with sickle cell disease/thalassemia, their families, and health care providers
 - **Sickle Cell Disease**
 - We created separate fact sheets and resource guides for providers and non-provider audiences, and distributed via a targeted mailing to healthcare providers, at conferences, via community based organizations, and on social media.
 - The targeted mailing was accompanied by a survey asking for physicians' opinions on the utility of these materials as well as their general knowledge of SCD. (Results from analyses of the survey data are detailed in Appendix 2: Evaluation Report.)
 - We developed a public service announcement on SCD that promoted blood donation by African American donors (particularly males); this short film was shown at movie theaters with high attendance by African American movie goers

in December 2013, and has been shown at conferences and via social media. This PSA was also evaluated using a survey (accessed on-line or in paper form), and results of analyses of these data are also available in the Evaluation Report.

- Our partners at UCSF Benioff Children’s Hospital Oakland re-worked and substantially improved their California Sickle Cell Disease website (casicklecell.org) to share information, research, and resources with people impacted by SCD in the state and those working with them.
 - We created Facebook and Twitter accounts targeted at Californians with or impacted by SCD, and disseminated messaging widely through these media.
 - We completed and published a journal article in Pediatric Blood & Cancer detailing the RuSH methodology and results for SCD. A second article in collaboration with Georgia is nearing completion, detailing findings on mortality in SCD in the RuSH data for the two states. We have completed analyses for a third publication on the relationship between mental health diagnoses found in RuSH SCD case data and hospital utilization; publication is pending first author development of the manuscript. We presented findings from analyses of the RuSH data to a variety of outside audiences, including the Newborn Screening and Genetic Testing Symposium (2014), the Annual Scientific Symposium in Sickle Cell Disease, the Sickle Cell Disease Foundation Association of America (2013 and 2014), the American Society of Hematology’s annual meeting (2014), the Children’s Hospital Oakland Research Institute Annual Meeting on Sickle Cell Disease (2013), and the Loma Linda Medical Center Annual Conference on Sickle Cell Disease (2013 and 2014).
 - We have provided technical assistance and advice to a number of colleagues developing grant proposals for sickle cell disease research using administrative data, and have participated in meetings with state health system policy makers to advise on problems in SCD care.
 - We conducted outreach and health promotion to local county departments of health as well as county jail systems, and a Public Health Institute event for Black History month. One or more members of our team was present and conducting health promotion/outreach at all of the outreach events to recruit racially and ethnically diverse donors and blood drives scheduled by Blood Centers of the Pacific during 2013 and 2014; our Community Engagement Coordinator also attended most of the meetings of the Northern California Sickle Cell Community Advisory Council.
- **Thalassemia**
- Our partners at UCSF Benioff Children’s Hospital Oakland developed social media presences similar to those created for SCD, and distributed wellness, health promotion and awareness messages.

- While we are confident that our extensive health promotion, outreach and dissemination efforts has had some impact on the prescription and uptake of hydroxyurea, blood transfusions and other complication prevention measures in sickle cell disease and on the diagnosis and treatment of previously undiagnosed cases of thalassemia, we do not have direct evidence of achievement.

2. Describe significant results of the project (positive or negative). Include both data results and other, less tangible results (ex. policy change, healthcare enhancement, etc.). (1-2 paragraphs)

We believe there are important and far-reaching beneficial results from the PHRESH project in California. Following on the work done in the RuSH project, PHRESH brought sickle cell disease and thalassemia into focus as public health problems. Meetings with policy makers had direct results, such as simplifying some criteria for continued enrollment into the state's Genetically Handicapped Persons Program and exemption of SCD and thalassemia from the managed care system being implemented throughout the California Children's Services program as the Affordable Care Act took effect. Evaluation and feedback from our health promotion efforts via the internet, social media and our SCD public service announcement informs us that our messages reached thousands of people in the state, most of whom (via social media and the casicklecell.org and thalassemia.com websites) were actively looking for more information about these disorders. Feedback from our mailings to providers tells us that these materials and information filled an important knowledge gap among non-hematologist healthcare providers. The printed materials, public service announcement, social media presences and websites created as a result of the project are sustainable and will continue to be made available to the public and providers as funding for distribution and support exist.

Additionally, the PHRESH project allowed for validation of the ground-breaking Registry and Surveillance System in Hemoglobinopathies. The two year effort to identify strengths and weaknesses in the data sources and methods used to conduct this pilot program was fruitful; we identified and collected data on additional confirmed cases, evaluated the utility of all data sources, improved the case definitions, made suggestions for future surveillance efforts and revised case counts. Going forward, California is now in an excellent position to develop a long-term surveillance system and the Validation Report created as the culmination of this work is available to other states or health districts that wish to establish a similar surveillance system for thalassemia, sickle cell disease or for other health conditions that may be identified in a similar way. Improved data based on this validation effort also have the potential to inform researchers, clinicians, health policy makers, drug developers and others in the future.

3. Describe the project-related activities that you think would be most beneficial to continue if years of future funding were available. (1-2 paragraphs)

Continued surveillance of the SCD and thalassemia populations in California, even without new data sources, would improve the utility of the data and accuracy of the conclusions drawn from it. We would pick up more cases over time and come to understand more about current complications and outcomes as well as the impact of current treatments at a population level. Such work would continue to inform and expand the impact on those with these disorders as well as those who work with them, policy makers, etc. Continued surveillance with improved and expanded data sources as described in the Validation Report would take this work a step further, allowing a finer grained look at the populations impacted by SCD and thalassemia, their complications and co-morbidities, treatments and outcomes. In particular in SCD, many new treatments and drug options will soon be available, and surveillance of

population outcomes could be of enormous benefit in understanding their uptake, effectiveness and any new complications or significant side effects. Increased/continued funding would also allow for increased health promotion and advocacy efforts in these disorders. There are opportunities to publish many additional manuscripts, conduct continued outreach activities, develop new materials and expand distribution of these and the original materials.

4. Provide a list of publications resulting from the project – those already published and those planned for the future.

COMPLETED Publications and Presentations

- Paulukonis ST, Harris WT, et al, "Population based surveillance in sickle cell disease: methods, findings and implications from the California registry and surveillance system in hemoglobinopathies project (RuSH)." *Pediatr Blood Cancer*. 2014 Dec.
- Paulukonis ST, Currier R, et al, "Impact of Immigration and Migration on Thalassemia Surveillance in California, 2004-2008" (presentation at the American Society of Hematology annual meeting, San Francisco, CA, December 2014).
- Paulukonis ST, Griffin T, et al, "Sickle Cell Disease Mortality in California and Georgia 2004-2008" (presentation at the American Society of Hematology annual meeting, San Francisco, CA December 2014).
- Feuchtbaum L, Rosenthal N, et al, "California Sickle Cell Disease Provider Survey: Assessment of Patient Management Approaches, Barriers to Care and Educational Needs" (presentation at the Association of Public Health Laboratories annual conference, Anaheim, CA, October, 2014).
- Hutchinson C, Rosenthal N, et al, "Challenges and Needs of Primary Care Providers Who Manage Care for Patients With Sickle Cell Disease" (presentation at the 42nd Annual Sickle Cell Disease Association of American Convention, Baltimore, MD, October, 2014).
- Hulihan MM, Feuchtbaum LF, et al, "State-based surveillance for selected hemoglobinopathies," *Genet Med*. 2014 Jul.
- Griffin T, Hulihan MM, et al, "Sickle Cell Disease Mortality in California and Georgia 2004-2008" (presentation at the Eighth Annual Foundation for Sickle Cell Disease Research Scientific Symposium, Miami, FL, April, 2014).
- Hutchinson C, Treadwell MT, et al, "Using community engagement and social media to raise SCD awareness" (presentation at the Eighth Annual Foundation for Sickle Cell Disease Research Scientific Symposium, Miami, FL, April, 2014).
- Paulukonis ST, Feuchtbaum LF, "Findings and Implications from the California RuSH Project" (plenary presentation at the Eighth Annual Sickle Cell Disease Research and Educational Symposium, Miami, FL, April, 2014.)
- Paulukonis ST, Feuchtbaum LF, "Results of California's Registry and Surveillance System for Hemoglobinopathies (RuSH): Sickle Cell Disease Case Counts, Outcomes and Healthcare Utilization" (presentation at the Eighth Annual Foundation for Sickle Cell Disease Research Scientific Symposium, Miami, FL, April, 2014).
- Treadwell MT, Paulukonis ST, et al, "Population-based Study of Mental Health Diagnoses and Utilization in Sickle Cell Disease" (presentation at the Eighth Annual Foundation for Sickle Cell Disease Research Scientific Symposium, Miami, FL, April, 2014).
- Paulukonis ST, "California PHRESH: Finding, describing and validating SCD cases" (presentation at the 4th Annual Advanced Workshop on Sickle Cell Disease at Children's Hospital Oakland Research Institute, Oakland, CA, October, 2013).
- Feuchtbaum L, Hutchinson C, "California's PHRESH Project" (presentation at the Second Annual Sickle Cell Symposium, Loma Linda University, Loma Linda, CA, September, 2013).

- Rosenthal N, Feuchtbaum LF, et al, “Long-Term Follow-Up of Patients with Hemoglobinopathy Diagnosed through the California Newborn Screening Program – Preliminary Findings” (presentation at the Association of Public Health Laboratories annual conference, Atlanta, GA, May, 2013).
- Hutchinson C, Paulukonis ST, “Research, Epidemiology and Surveillance in Thalassemia” (presentation at the Fifth Annual Thalassemia Support Foundation Conference, Los Angeles, CA, April, 2013).

PLANNED/IN DEVELOPMENT PUBLICATIONS AND PRESENTATIONS

- Population-based Study of Mental Health Diagnoses and Utilization in Sickle Cell Disease (publication).
- Defining the new face of sickle cell disease mortality using a population-based surveillance system (publication).
- Impact of Immigration and Migration on Thalassemia Surveillance in California, 2004-2008 (publication).
- Emergency Department Utilization among the California RuSH Cohort of Sickle Cell Disease Patients, 2005-2013 (abstract presentation at the Ninth Annual Foundation for Sickle Cell Disease Research Scientific Symposium and publication).
- Thalassemia management patterns and challenges in California: findings from the Thalassemia Provider Survey

5. Assess the challenges and opportunities of the pilot project including a description of the problems encountered, lessons learned, potential improvements, etc. (2-3 paragraphs)

There were few significant challenges to completing the objectives of the project beyond a significant budget cut in Year 2 and the ending of the project at close of Year 2, eliminating a hoped-for third year for continued activities. Funding at the anticipated levels would have allowed, as above (Question 3), for expanded health promotion and a commensurate increase in activities that impact the affected populations (example, more opportunities to work with county health departments) as well as continued and expanded surveillance in SCD and thalassemia. Other challenges encountered in the process of the validation of RuSH methods include a lack of consistency of staff, limited capacity for increased validation data collection, and limited involvement and expertise for one of our advisory groups.

- Consistency of staff – a primary purpose of the PHRESH project was validation of the RuSH data and methodology. However, there was a break in funding between the end of the RuSH project and the beginning of the PHRESH project. All key staff on the RuSH project left California’s Genetic Disease Screening Program for other employment during this time. The project director returned at commencement of the PHRESH project, but was part-time for most of of Year -1 while finishing another project. RuSH’s database developer and the creator of the linkage algorithm used to create the original RuSH data set moved on permanently, and his absence, despite extensive documentation, meant a steep learning curve for the validation effort.
- Obtaining patient-level data with identifying information is a time-consuming effort, as is establishing sub-contracts with state academic medical centers. For the former, state as well as institutional IRB approval is needed, PI and staff time are required, and cases must be identified and abstracted; for the latter, months can pass in the establishment of contracts and letters of agreement. Because of this and because of limited funding, we stopped collection of validation

data in February of 2014 although more data would have benefitted the validation efforts and would have contributed to an increased case count and better understanding of the populations affected. A three year project would have been preferable to include more time for data collection and for analysis and dissemination of the final data.

- As detailed in the Evaluation Report, the gathering and engagement of community members for our thalassemia advisory group was a challenge. The establishment of this group had the potential to have a significant impact on advocacy around thalassemia in the state, but a number of factors prevented a strong level of commitment from the community impacted. One of these factors was reluctance among members of the affected community to come forth and fully engage with the Advisory Committee. Additional time and effort, perhaps through more frequent meetings and tangible projects, may have improved this level of engagement and allowed for the development of stronger partnerships.

Attachments:

Appendix 1: Validation Report

Appendix 2: Evaluation Report