

Peer Network Resource Centers for New RUSP Disorders
October 18, 2016

Sikha: Thank you everyone for joining us this afternoon. As noted previously, this webinar will be recorded and archived. Following the presentation, there will be an opportunity for questions and answers. This RFP is an opportunity for entities to apply to receive funding to serve as a Peer Network Resource Center for new [inaudible 00:00:23] disorders. This is a component of a new disorders implementation project cooperative agreement awarded to APHL by HRSA on September 1, 2016. APHL collaborates with the Colorado School of Public Health to execute all activities of the NewSTEPs program, and for the purposes of this cooperative agreement we are also entering into formal partnerships with Baby's First Test as well as with the newborn screening translational research network to conduct long-term follow-up and educational components of the goals.

The goals of this cooperative agreement are four-fold. The first goal is to assess needs and allocate resources for new condition integration into newborn screening systems. The second goal is to provide educational resources and technical assistance consistent with getting funding increases and authority to implement screening. The third goal is to support the implementation of laboratory and follow-up screening for state newborn screening programs enabling them to achieve full implementation of newborn screening for at least one of the new conditions by September of 2018. The fourth and final goal is to develop and disseminate community, parent and provider educational information.

This slide demonstrates the state of newborn screening currently as of October 2016 for Pompe, MPS-1 and X-ALD newborn screening. The majority of programs in the United States are not currently screening for these newest disorders [inaudible 00:02:11]. For Pompe disease which was added to the recommended uniform screening panel in March 2015 there are five states currently offering universal newborn screening. For MPS-1 and X-ALD which were both added to the RUSP in February of 2016, there are four and three states respectively offering universal newborn screening for those disorders.

The RFP schedule for the specific RFP is that the RFP was issued on October 11th. The PA webinars are taking place today. A letter of intent is due by 5:00 pm, eastern time tomorrow, Wednesday, October 19th. APHL requests that all prospective applicants submit a brief email statement indicating their intent to apply to submit a proposal by no later than the RFP due date. The RFP due date is November 18th at 5:00 pm, eastern time. We want to note again that potential applicants must include the name of the organization and the individual that will submit the proposal in this email, including the letter of intent. The prospective bidder should email that statement to

me, that's Sikha Singh as well as to the NewSTEPS email address which will be found at the end of this webinar, also within the RFP document. We want to note that while you're required to submit a letter of intent if you intend of responding to the RFP, that submission of a letter of intent is not binding, so you're not required to respond to the RFP even if you do submit a letter of intent.

Any modifications to the schedule as well as to the RFP questions and language that may be made will be communicated on www.aphl.org/rfp.

Objectives of this RFP is to fund entities that will serve as Peer Network Resource Centers. What does this mean? PNRs will be comprised of a network of experts that can assist newborn screening programs in implementing screening for MPS-1, Pompe and/or X-ALD. They may also serve as subject matter experts on laboratory techniques as well as serving as content experts for follow-up procedures. The Peer Network Resource Centers may offer training on new disorder newborn screening implementation as well as offer first and/or second tier testing.

The eligibility criteria to serve as a Peer Network Resource Center are three-fold. In order to apply, you must meet at least two of the three criteria which I will read to you momentarily. The first criteria for eligibility is the ability to serve as a content area expert and to provide technical assistance to state newborn screening programs on the implementation of laboratory techniques, follow-up procedures and other activities specifically associated with the implementation of screening for Pompe and/or MPS-1 and/or X-ALD. This technical assistance may include hosting on-site visits by other newborn screening programs to receive training and mentorship on laboratory practices and/or follow-up processes associated with implementation and screening for at least one of the new conditions.

Eligibility criteria two of three, and let me remind you again, that programs applying for this must meet at least two of these three criteria. The second criteria is the ability to offer first tier screening for the new disorders for other state newborn screening programs thereby demonstrating capacity to increase the number of specimens screened for the selected disorder. Screening for other programs could be short term in order to facilitate earlier testing by a state program, or it could be long term with the intent of providing screening results for a given disorder on an ongoing basis.

First tier screening in a peer network resource laboratory may be selected on a short term basis by state programs who are unable to complete the laboratory validation and set up but are otherwise ready to implement the screen. Covering costs associated with a peer network resource laboratory, performing laboratory screening for another program will be the responsibility of the requesting program. Funding provided through this award mechanism is intended to support the development of the infrastructure within the Peer Network Resource Center to allow additional testing.

The final eligibility criteria is that the Peer Network Resource Center would offer second tier or confirmatory testing in the form of molecular or next generation

sequencing following a positive tier newborn screen and other newborn screening program. Currently, the low volume of second tier of screening in newborn screening programs makes it impractical to establish confirmatory capabilities in all laboratories. Covering the costs again associated with the Peer Network Resource laboratory performing the screening for another program will be the responsibility of the requesting program. Funding provided through this award mechanism is intended to support the development of the infrastructure to allow additional testing.

All of this language, I want to add, is found within the RFP which is found on aphl.org/rfp.

In completing your application, you must submit the following three sections which are further explained again in the RFP under the response requirement. Section one is the responses to questions. These questions are when did or will your program initiate screening for each disorder and what methodology either is or will be used. The second question is describe your program's capabilities to provide technical assistance, and finally, describe your laboratory's capability and maximum capacity to provide first tier and/or second tier screening for each disorder. You'll find that the questions here in section one are consistent with the eligibility criteria we just discussed.

Section two, another required section, requires a budget and budget justification of up to \$60,000 per year for two years. Funds can be used to provide technical assistance. Funds can also be used to host training. We want to mention that no travel costs will be incurred to the Peer Network Resource Center to host the trainings. APHL will cover those travel related costs separately. However, if you are capable of hosting trainings and this is a function of the technical assistance you wish to offer, please include in your budget consumable related costs that would occur by virtue of conducting those trainings. There is additional details on this, again, in the RFP.

Another way funding can be used is to establish an infrastructure to perform first or second tier screens for one or more of the disorders. We do want to note that the appropriateness of the budget proposed will be evaluated based on the number of disorders screened for by the Peer Network Resource Center. Additionally, the budget justification should be detailed describing how you arrived at the cost.

The final required component of the application is a sustainability plan. Again, this is mandatory and in it you must explain how the approach that you proposed will be sustained following the funding period.

Now I'm going to hand it over to Dr. Yvonne Kellar-Guenther, the evaluator for NewSTEPS, who will discuss with you the evaluation criteria for this RFP. Yvonne, if you want to dial star seven to unmute.

Yvonne: Yes, thank you. The evaluation criteria we have to take all of the applications into account, so we're going to look at it a holistically. We've included what the rubric is so

you can actually see the rubric, how we use to score, but we have to look at each piece individually. One of the sections that you'll be scored on is your ability to screen for the different conditions. Each condition has its own scoring. It's max of 50 points for each condition. They will not be combined, but we'll look to see who's strong in each condition. If someone is strong in two conditions, they will be overall scored a little higher than someone who's strong in one condition.

Again, if someone is very strong in one condition and no one else is as strong, then they could be the one that gets chosen to help with that condition. It's very much a holistic process where we have to look at all the people who have submitted and what support we can offer to the awardees for the states.

For the condition, when we look at condition, for each condition separately we look at your experience screening. If you are screening, how long you've been screening. We look at your ability to provide condition specific TA, so your lab experts, your follow-up experts, your clinical experts. We look at whether you have education materials already or you have access to education materials. We look at whether you already have a way to measure the impact of education materials, and then lastly per condition we look at your ability to report on the condition screened as well as if you already have a sense of cost for that. Next slide, please.

The other pieces that we look at besides each individual condition, then you we look at your TA experience in general. Again, we'll look for the TA piece we'll look for sites that have high TA experience. We're very interested in your team and how your team works together, so explaining how your team communicates, explaining what everyone has access to and how you work together, if there is a referral system already in place, and then if there's a system in place for the diagnostic data. We look at also your team's experience teaching others, so if you've trained others in their laboratory or in their follow-up program or you've visited other states, if you've had other states visit you. Also, it should be noted that throughout we don't really have a multiplier for experience with more disorders, except for here under the testing experience ...

Sorry, we have another section which is testing experience, here we look at ... If you have experience conducting first tier testing and second tier and next gen sequencing testing, we look in this section at your informatic capabilities, if you have experience reporting your informatic capabilities, if you have experience turning around reports for others. Here we do have a multiplier if you have more experience with more disorders, you would get more points for one of the places that we score you.

The final section is the budget and sustainability plan. We'll look at this also, but again, it will kind of go into a score for your site. This is looking at sustainability. How are you going to sustain? If you have ongoing TA efforts, how those are being sustained. If you have ongoing or you're screening for others, how you're going to sustain those efforts, and how feasible it is. Finally we'll look at your budget, if the budget items that are listed relate to the activities that you have in your plan for what you're offering, and if you are able to justify your costs.

Next slide.

Again, we look at each piece separately so we'll look at your ability to screen or provide TA for MPS-1, for X-ALD, all the different conditions. We'll look at the TA experience separately, the testing experience separately, and then your budget and sustainability will kind of go into your overall picture of how you are as a state. We need to look across everyone who applies and try to make sure that we choose sites that provide holistic coverage for the states that we will be funding.

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Now we will open it up for any questions.

Sikha: If you have a question please dial star 7 to unmute yourself. If you prefer you can type in questions into the chat box in the lower left hand side of your screen.

Following the webinar, if you have any questions you can reach out to any of the addresses listed on the slide that you're looking at newsteps@aphl.org or sikha.singh@aphl.org. We want to remind everybody that any questions raised or modifications made to the RFP will be shared on www.aphl.org/rfp. All letters of intent are due tomorrow, Wednesday October 19th by 5 PM, eastern time.

We do have a question in the chat box. The question is, does the confirmatory testing have to be molecular-based? The second tier confirmatory testing, does it have to be molecular-based? The response is, no it does not have to be, if there is a methodology that you would like to use to confirm or perform second tier screening for any of the three disorders, you are welcome to do that. Again, just list it out in your budget, the costs incurred and the budget justification accordingly.

We have another question in the chat box. Is it anticipated that the trainees only travel to the Peer Network Resource Center and not the subject matter experts from the Peep Network Resource Center travel to the training laboratories. It is up to the program. We have to RFPs out right now, one it for the PNRC, the PNRC, the Peer Network Resource Centers who are provided funding will have the opportunity to host trainee's programs at their own laboratories to receive training, however the states who are also provided funding to perform implementation in any of the three disorders have the opportunity to identify mechanisms for their training to occur. They may choose in their application to invite experts from within the Peer Network Resource Centers to travel to their laboratories. That was a very long answer to a rather short question but it is reciprocal, it is something that can be customized, and it's not limited to just one direction.

If there is any other questions, please dial star 7 to unmute your phone. We have another question in the interim, in the chat box asking if it is acceptable to include staff time in the training as a consumable. It is, and you don't have to list it as a

consumable, you can in fact introduce staff time consistent with the training that you wish to host. Yes, you may include staff time.

If there is any additional questions please dial star 7 to unmute or to list them in the chat box in the bottom left of your screen.

Hearing and seeing no additional questions, we will end this webinar. Again, if you have any questions, feel free to reach out, this webinar has been recorded and will be posted on the website. Please note that the letter of intent is due by 5 o'clock pm, eastern time tomorrow, October 19th.

Thank you.