



National PKU Alliance

Home Phe Monitor Research Challenge
Advancing the Treatment for PKU

Support for a Home Phe Monitor

“Over the past 10 years, major advances have occurred in PKU scientific understanding and therapeutic research, but the key bottleneck to addressing care is in evaluating blood phenylalanine (Phe) levels. This is similar to diabetics and glucose levels. Evaluating Phe levels is still in the dark scientific ages and I personally believe the absolute highest priority is to develop a home Phe monitor.”

Ray Stevens, Ph.D.

Scripps Research Institute, La Jolla, CA

Support for a Home Phe Monitor

“Real time, point-of-care Phe monitoring would provide more opportunity to engage patients in their own care.” – *American College of Medical Genetics and Genomics*

“The European Society of PKU fully supports the development of a home Phe monitor. Such a tool would lead to better compliance.” – *Eric Lange, ESPKU President*

Support for a Home Phe Monitor

“The development of a home device will address the most important gap in the current treatment strategy for PKU patients” –

*Charles Scriver, M.D. and
Christineh Sarkissian, Ph.D.
McGill University*

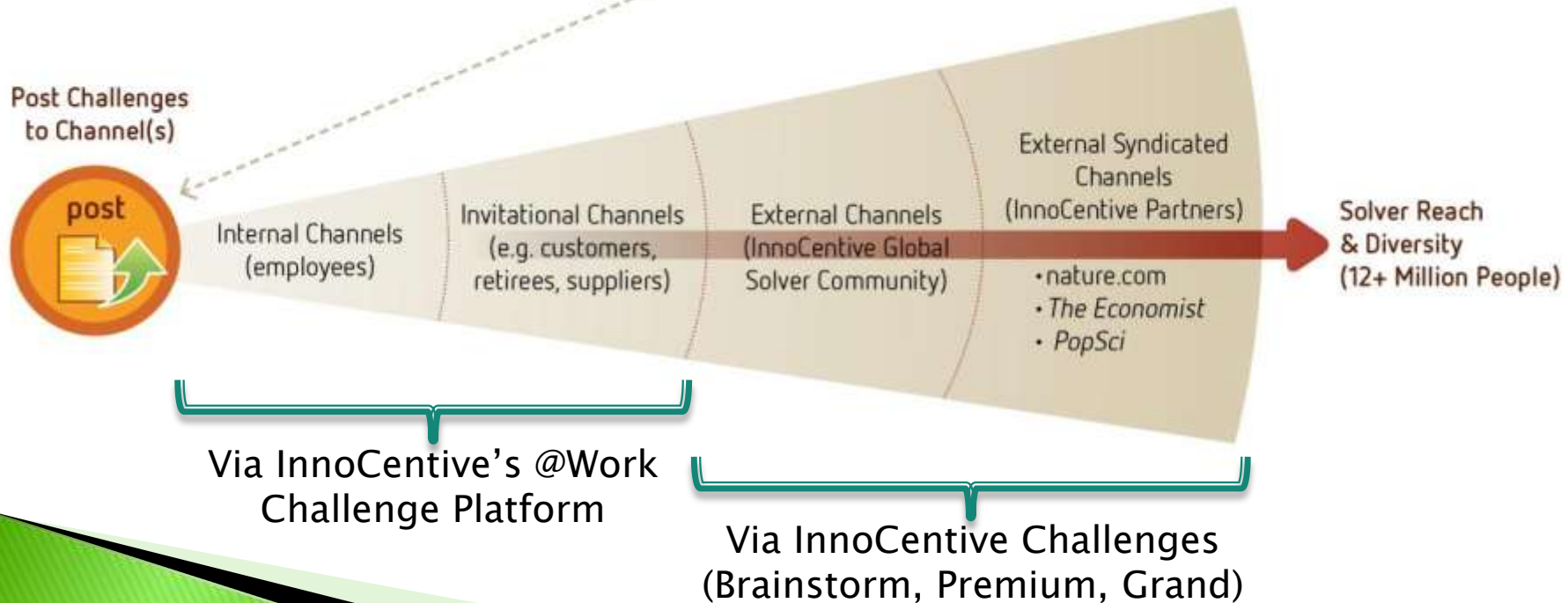
NPKUA partners with InnoCentive, Inc. & The Peter Cundill Foundation

- ❖ In 2013 the NPKUA reached out to InnoCentive, Inc. to help challenge the scientific and technical community around the world on the development of a Home Phe Monitor
- ❖ NPKUA & InnoCentive worked together to bring funding from The Cundill Foundation in Toronto to help support the project
- ❖ Together the NPKUA and Cundill Foundation have provided \$776,000 to support the Home Phe Monitor Challenge Project

InnoCentive Process

- ▶ **Challenge Driven Innovation – using cash prizes and challenge mechanisms to incentivize the development of innovative solutions from potential solver across the world.**

How the Challenge Process Works



InnoCentive Challenge Facts & Stats

Total Registered Solvers:	Over 300,000 from nearly 200 countries
Total Solver Reach:	13+ million through our partners
Total Challenges Posted:	1,700+ External Challenges & thousands of Internal Challenges (employee-facing)
Project Rooms Opened to Date:	475,000+
Total Solution Submissions:	37,000+
Total Awards Given:	1,400+
Total Award Dollars Posted:	\$39+ million
Range of awards:	\$5,000 to \$1+ million
Average Award Rate:	74%

Numbers valid as of March 1, 2013

InnoCentive Solver Community



InnoCentive Customers & Partners (sampling)

Commercial Customers



Government Customers



Nonprofit Customers



Strategic Partners



Booz | Allen | Hamilton

delivering results that endure



Challenge Statement's Criteria for Solutions

- ▶ **Ease of use in home by lay people**
- ▶ **Work with blood sample from finger stick**
- ▶ **Use existing readout technologies**
- ▶ **Amenable to manufacturing and commercialization at low costs per use**
- ▶ **Sensitivity, precision, & resolving power**
- ▶ **Specificity for phe**
- ▶ **Speed for analysis**
- ▶ **Bonuses if able to read <1 mg/dL & other metabolic analytes**

Three Challenge Phases

- ▶ **Phase I** – *Theoretical Challenge* opened to the world on how your technology would meet our specifications. Up to **8 awards of \$10K** each to be made to Solvers selected to move forward.
- ▶ **Phase II** – *A Three Milestone Challenge*: (1) to produced a more detailed Project Plan on how the Solver would demonstrate proof of concept for their technology – **6 awards for \$10K each** (2) provide test results from bench top prototype, **3 awards for \$50K each** & (3) demonstrate technology with whole blood sample from PKU patients – **1 award for \$200K** to produce a manufacturable & commercializable prototype.
- ▶ **Phase III** – *An eRequest for Partners* that would manufacture and commercialize the product.

Expert Review Panel

InnoCentive assisted us in Sourcing an expert review panel:

- ▶ Identify skill sets required to evaluate proposals
- ▶ Identify & recruit thought leaders:
 - NPKUA existing network
 - Commercial assay / platform providers
 - Literature review
 - InnoCentive Solvers with track record
- ▶ Early access to technology development is a powerful incentive!

Progress to Date

- ▶ Phase I Theoretical Challenge posted in **October 2013**
- ▶ 650 hits on InnoCentive Challenge posting
- ▶ 128 solutions received from Solvers located across the world (7 countries) **December 2013**
- ▶ Solutions narrowed to 30 for more in-depth review by Panel of Experts (15) **Jan – Mar 2014**
- ▶ Nine Solvers selected by Panel of Experts to enter Phase II for a more detailed project development plan **April 2014**

Progress to Date (continued)

- ▶ **\$10K Awards made to successful Phase I Solvers after verification process **May 2014****
- ▶ **Phase II Challenge Statement submitted to Solvers **June 2014****
- ▶ **Phase II detailed project development plans from Solvers due **July 15, 2014****

Phase II Challenge

- ▶ **There are three major milestones:**
 - **Milestone #1** – a detailed project plan including methods, materials, schedule, resources, & risks
 - Six awards at \$10K each
 - **Milestone #2** – demonstrate proof of concept test data, description of assay technology, materials, methods, and instruments
 - Three awards at \$50K each
 - **Milestone #3** – verification of proof of concept using clinical samples from PKU patients blood.
 - One award at \$200K

Future Project Goals

- ▶ **Evaluation of Milestone #1:** Solvers' Detailed Plans & Selection of up to 6 Solvers for an additional \$10K Award each for Milestone #2 **September 2014.**
- ▶ **Evaluation of Milestone #2:** Solvers' Proof of Concept test data & Selection of 3 Solvers for \$50K Award each **February 2015**
- ▶ **Evaluation of Milestone #3:** Solvers' verification of Proof of Concept with PKU patients' whole blood sample
- ▶ **Select one of the Solvers to move forward to a manufacturable & commercializable prototype with a \$200K Award. July 2015**

Eventual Goal

- ▶ **To have an affordable, compact, and easy to use Home Phe Monitor**
- ▶ **That will allow multiple readings per day or week if necessary to monitor blood phe levels**
- ▶ **Thus giving us information to better regulate diet and phe levels and avoid big swings in blood levels of phe on a daily or weekly basis**
- ▶ **But a major caveat here is that there is no guarantee that we will be successful....but we will have at least given it our best shot!!**

Sources of Funds

- ▶ Tia 4 Tuxes II Gala **\$307,350.**
- ▶ The Cundill Foundation **\$468,750.**
- ▶ **Grand Total** **\$776,000.**

Uses of Funds

▶ Cash Awards to Solvers	\$490,000.
▶ Research Costs	\$ 70,000.
▶ Expert Panelists' Stipend	\$ 15,000.
▶ InnoCentive Fees & Admin. Costs	<u>\$122,000.</u>
	\$697,000.
▶ Contingencies	\$ 79,000.
Total Expenses	\$ 776,000

Thanks so much for your attention

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