Crowdfunding for rare diseases

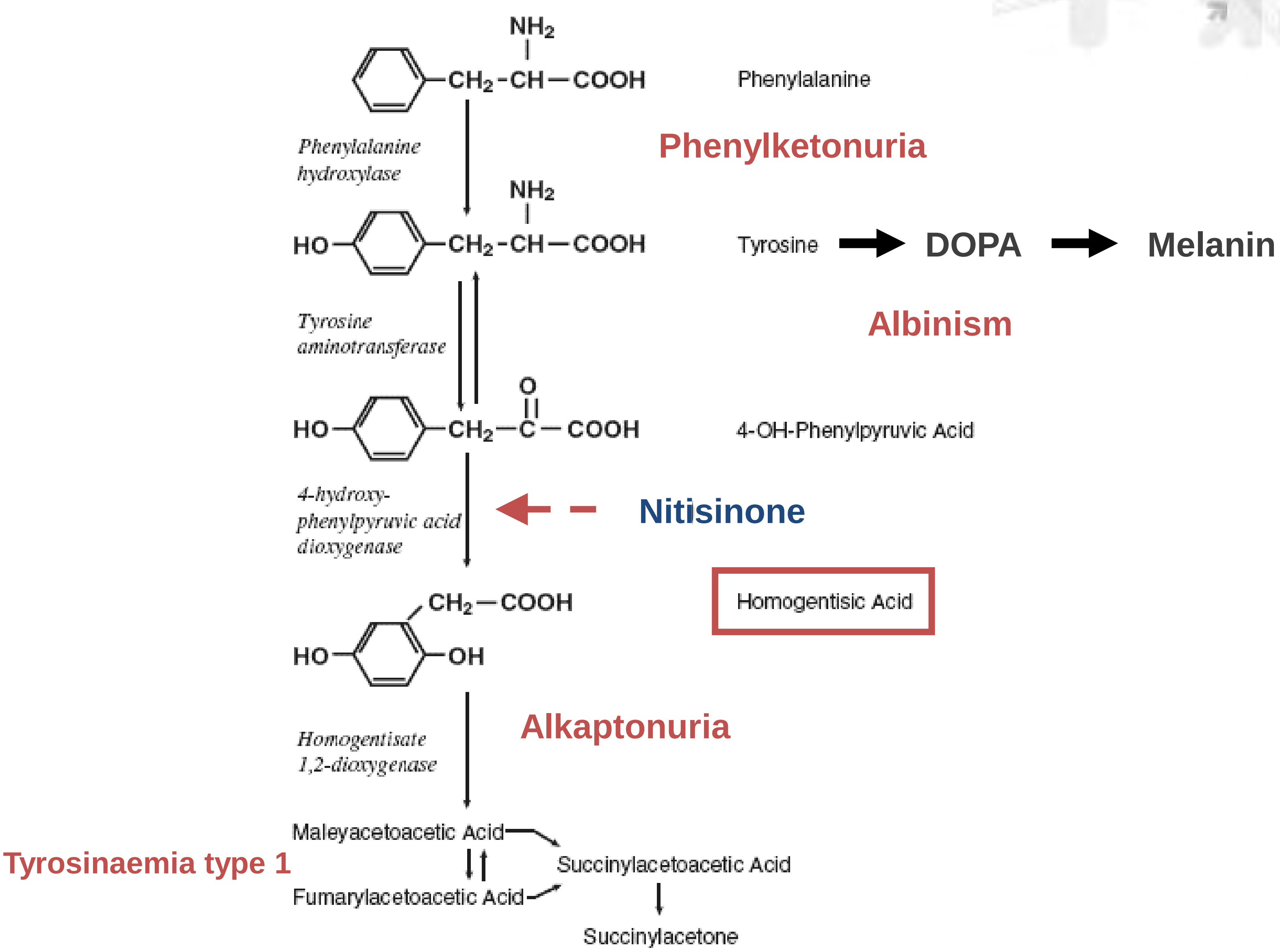
Lessons from AKU and Findacure

Dr Nicolas Sireau
Chairman and CEO, AKU Society
Chairman and Co-founder, Findacure

About AKU
(Black Bone Disease)

How AKU affects patients

Metabolic pathway



Phenylalanine

Phenylalanine hydroxylase

Phenylketonuria

Tyrosine → **DOPA** → **Melanin**

Albinism

Tyrosine aminotransferase

4-OH-Phenylpyruvic Acid

4-hydroxy-phenylpyruvic acid dioxygenase

← – **Nitisinone**

Homogentisic Acid

Homogentisate 1,2-dioxygenase

Alkaptonuria

Maleyacetoacetic Acid

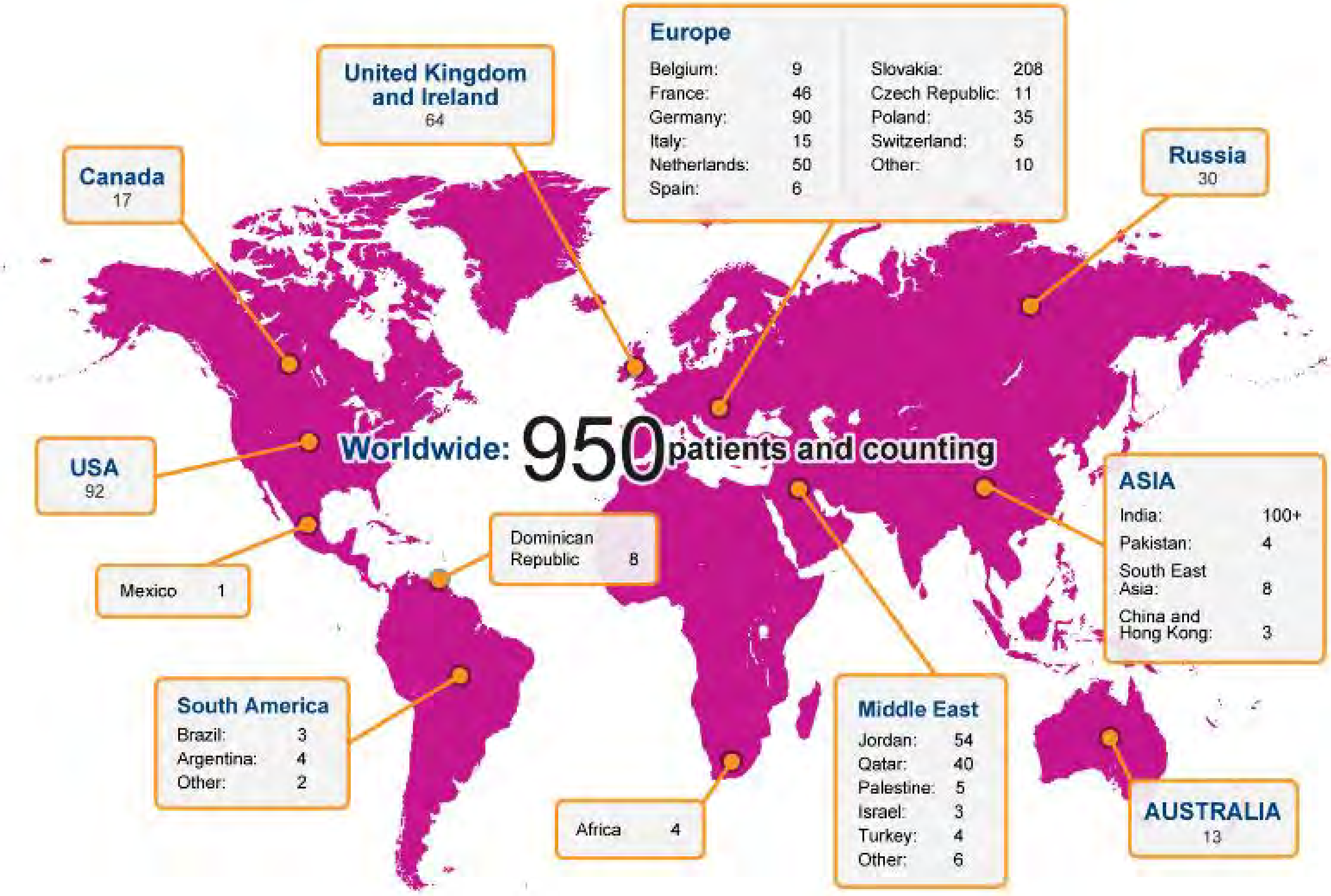
Fumarylacetoacetic Acid

Succinylacetoacetic Acid

Succinylacetone

Tyrosinaemia type 1

A global patient movement



Worldwide: 950 patients and counting

Canada
17

USA
92

Mexico	1

United Kingdom and Ireland
64

Europe

Belgium:	9	Slovakia:	208
France:	46	Czech Republic:	11
Germany:	90	Poland:	35
Italy:	15	Switzerland:	5
Netherlands:	50	Other:	10
Spain:	6		

Russia
30

Dominican Republic	8

South America

Brazil:	3
Argentina:	4
Other:	2

Africa	4

Middle East

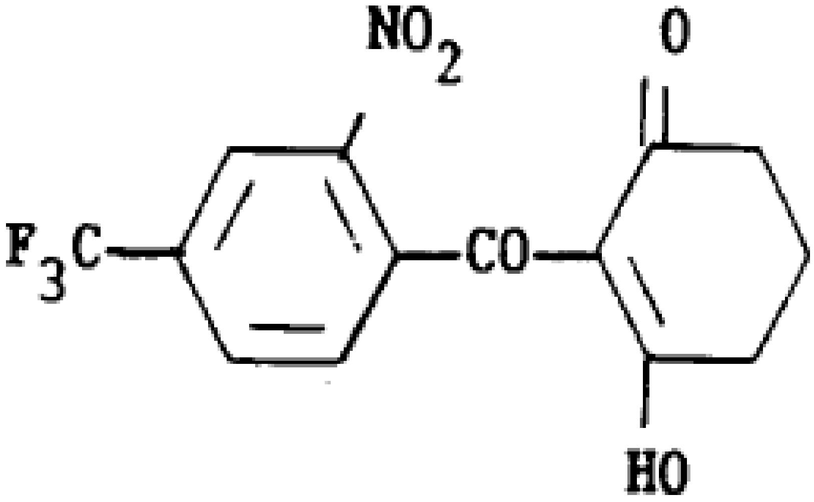
Jordan:	54
Qatar:	40
Palestine:	5
Israel:	3
Turkey:	4
Other:	6

ASIA

India:	100+
Pakistan:	4
South East Asia:	8
China and Hong Kong:	3

AUSTRALIA
13

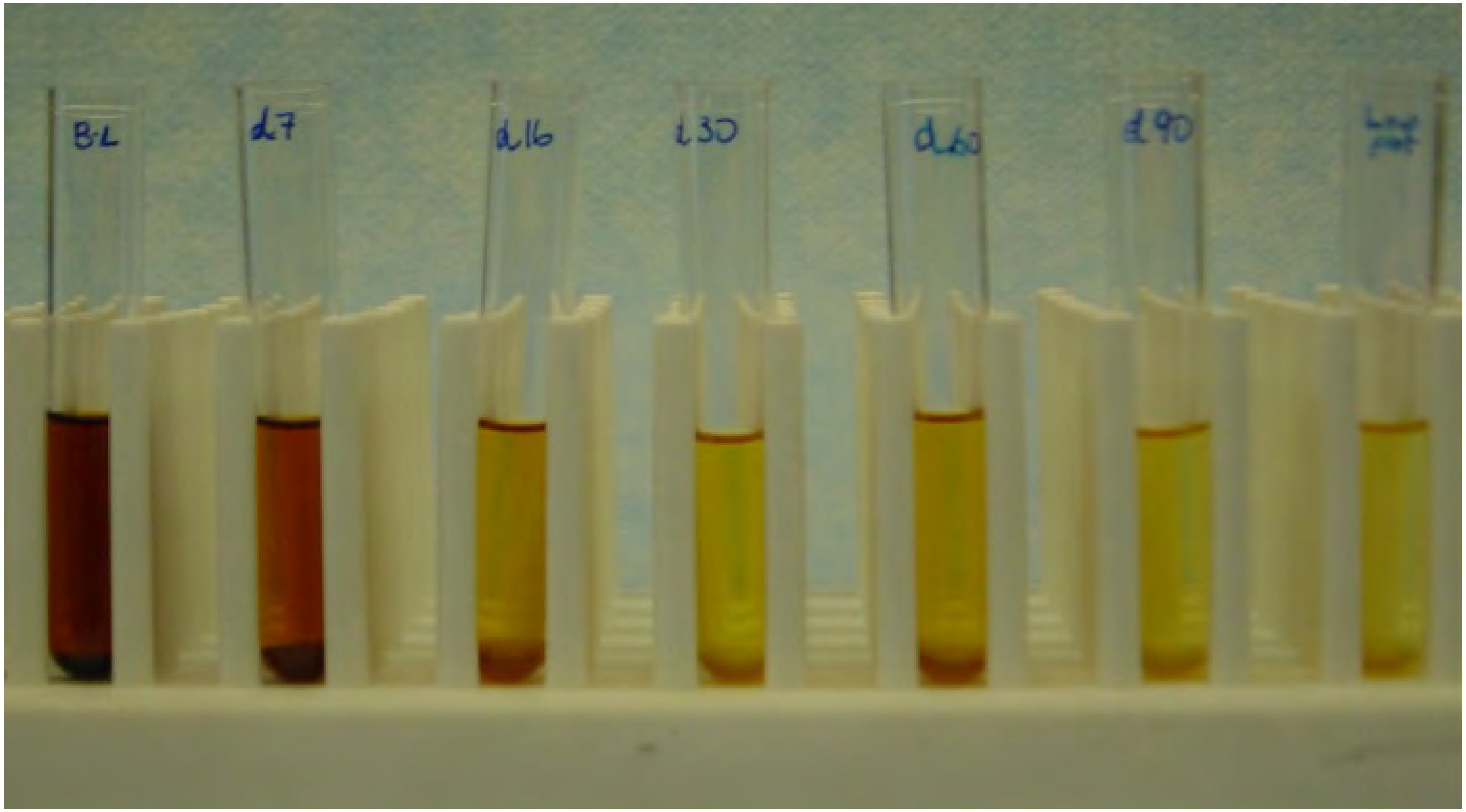
The treatment we were crowdfunding for: Nitisinone



Nitisinone reduces homogentisic acid by

95%

Why this drug is so promising



The crowdfunding campaign: key message and action

HELP US CURE BLACK BONE DISEASE

AKU patient Ann Kerrigan

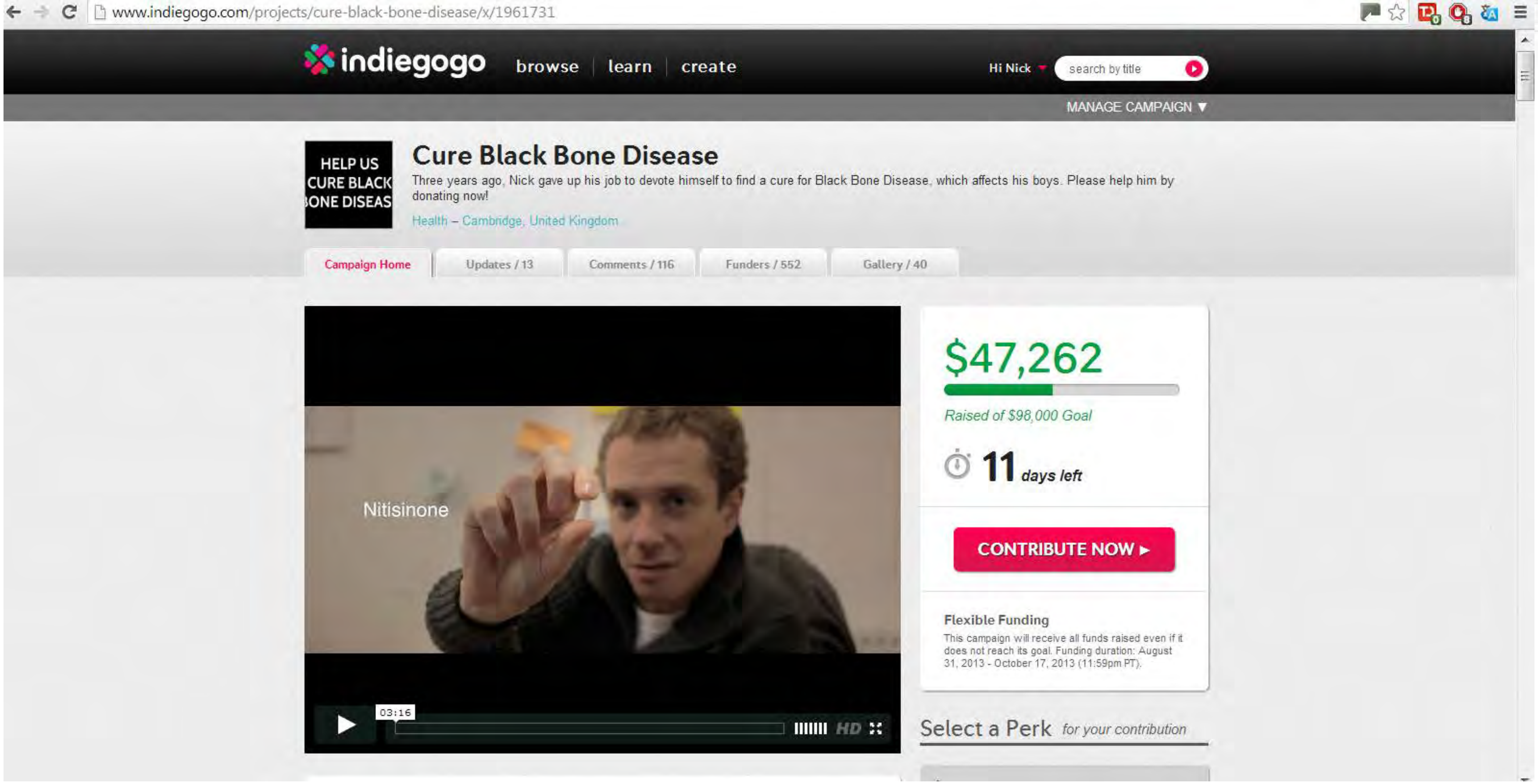
B M John Rajkumar,

AKU patient and head of the 500,000-strong Tamil Nadu Gypsy Society

Prof Ranganath,
Coordinator of DevelopAKUre

The AKU Society team

The campaign:
Indiegogo



Cure Black Bone Disease

Three years ago, Nick gave up his job to devote himself to find a cure for Black Bone Disease, which affects his boys. Please help him by donating now!

Health – Cambridge, United Kingdom

$47,262

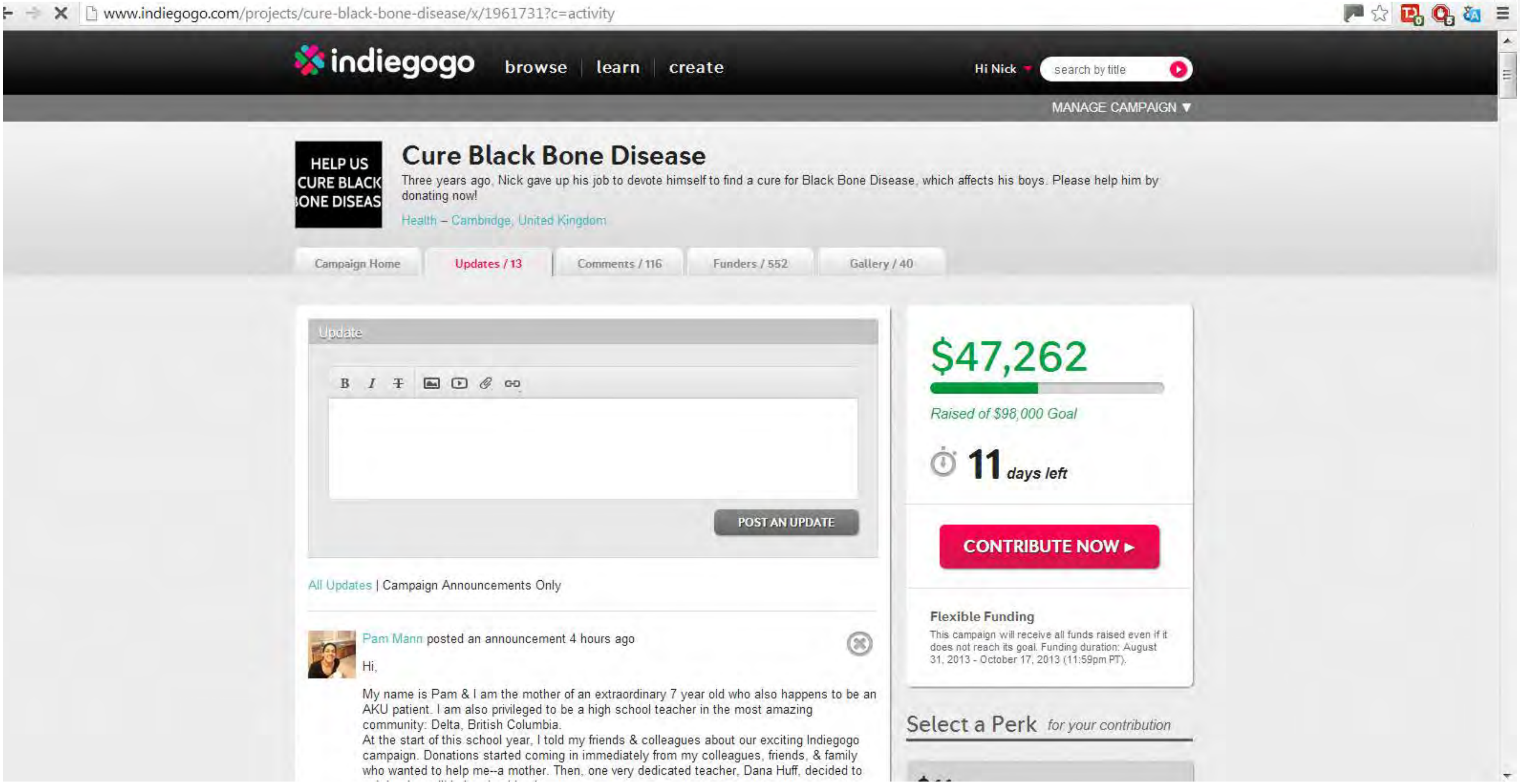
Raised of $98,000 Goal

11 days left

Flexible Funding

This campaign will receive all funds raised even if it does not reach its goal. Funding duration: August 31, 2013 - October 17, 2013 (11:59pm PT).

Select a Perk *for your contribution*



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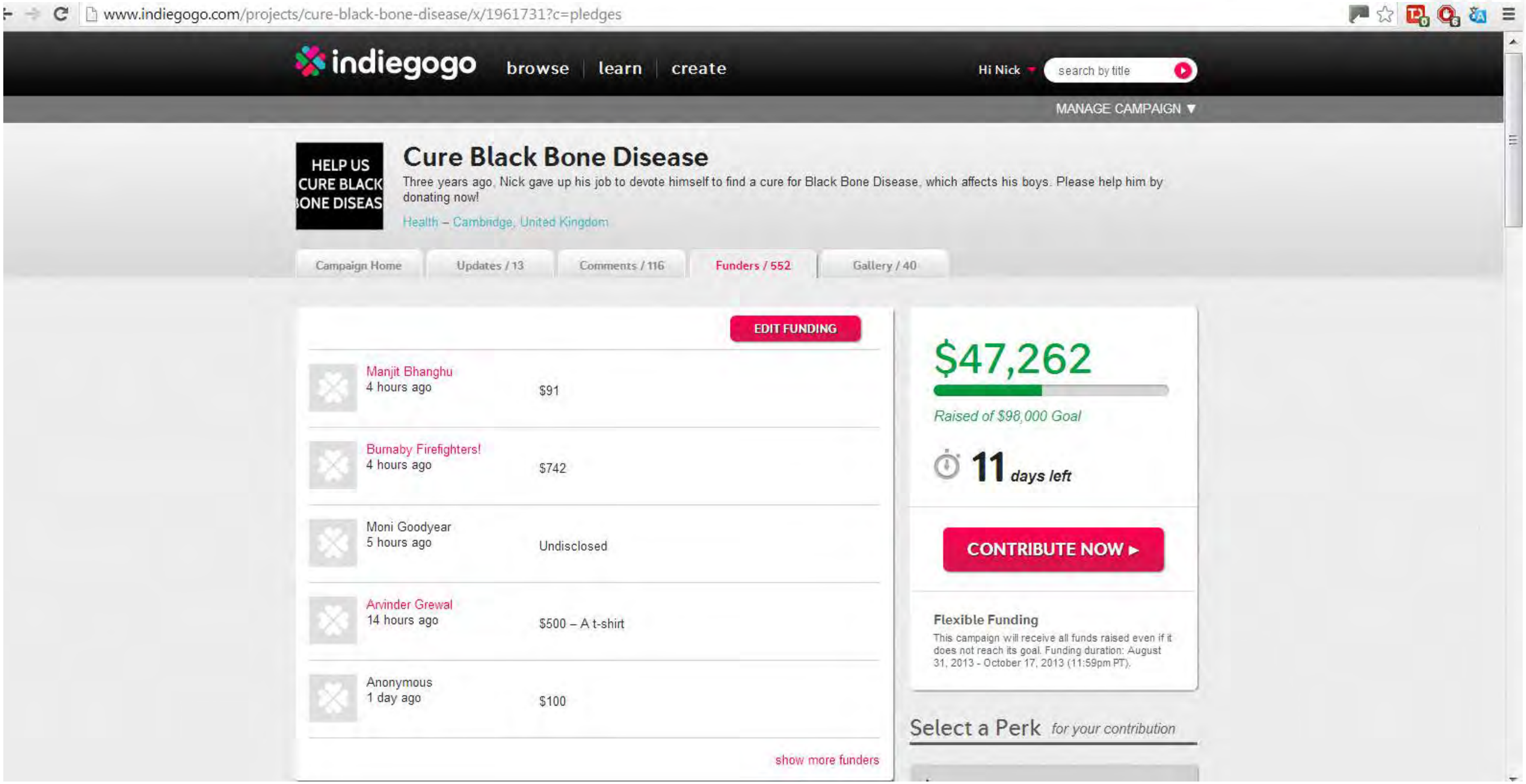
All Updates | Campaign Announcements Only

Pam Mann posted an announcement 4 hours ago

Hi,

My name is Pam & I am the mother of an extraordinary 7 year old who also happens to be an AKU patient. I am also privileged to be a high school teacher in the most amazing community: Delta, British Columbia.
At the start of this school year, I told my friends & colleagues about our exciting Indiegogo campaign. Donations started coming in immediately from my colleagues, friends, & family who wanted to help me--a mother. Then, one very dedicated teacher, Dana Huff, decided to



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Health – Cambridge, United Kingdom

Manjit Bhanghu 4 hours ago	$91
Burnaby Firefighters! 4 hours ago	$742
Moni Goodyear 5 hours ago	Undisclosed
Arvinder Grewal 14 hours ago	$500 – A t-shirt
Anonymous 1 day ago	$100

$47,262

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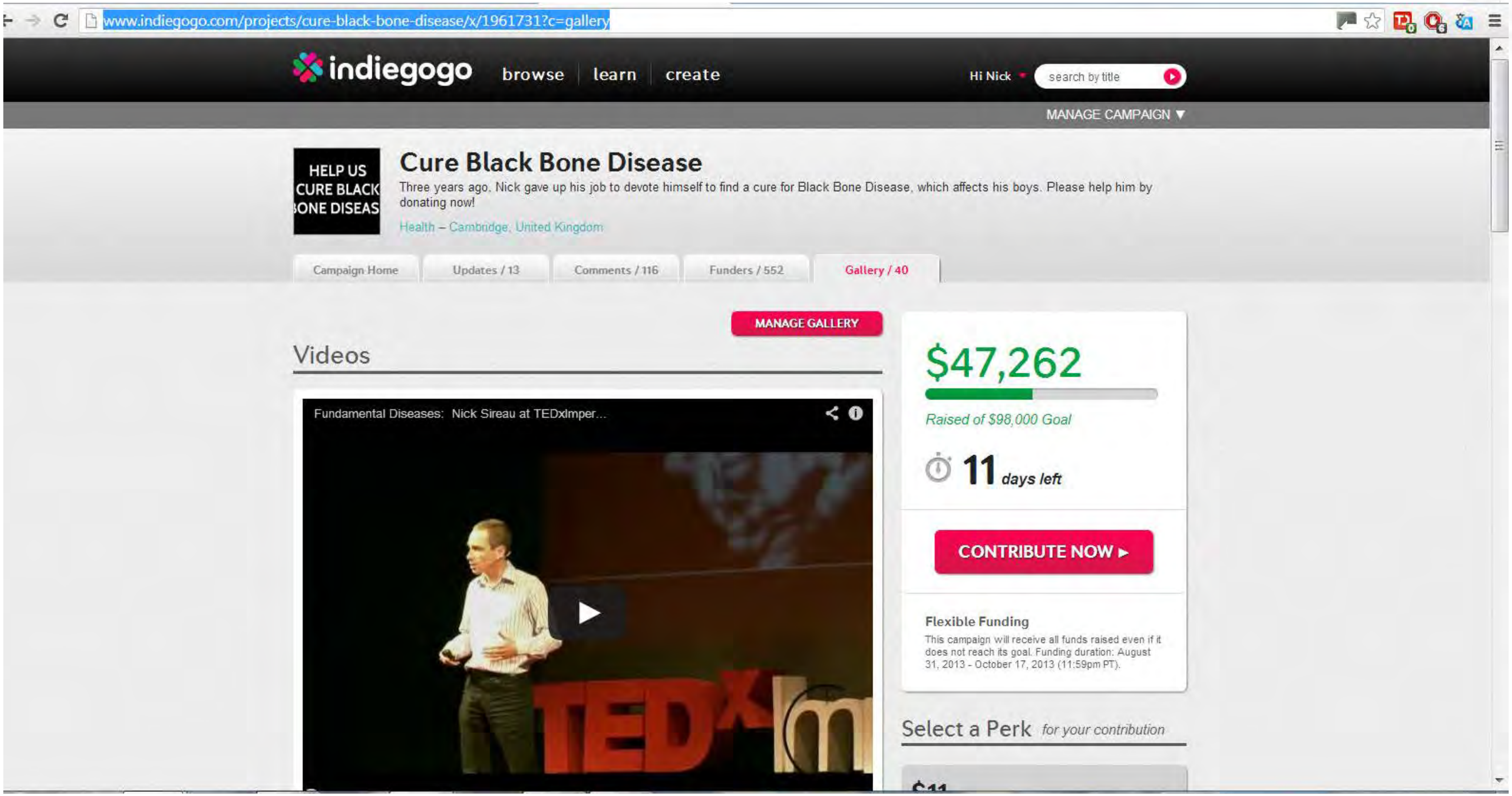

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Health – Cambridge, United Kingdom

Videos

Fundamental Diseases: Nick Sireau at TEDxImper...

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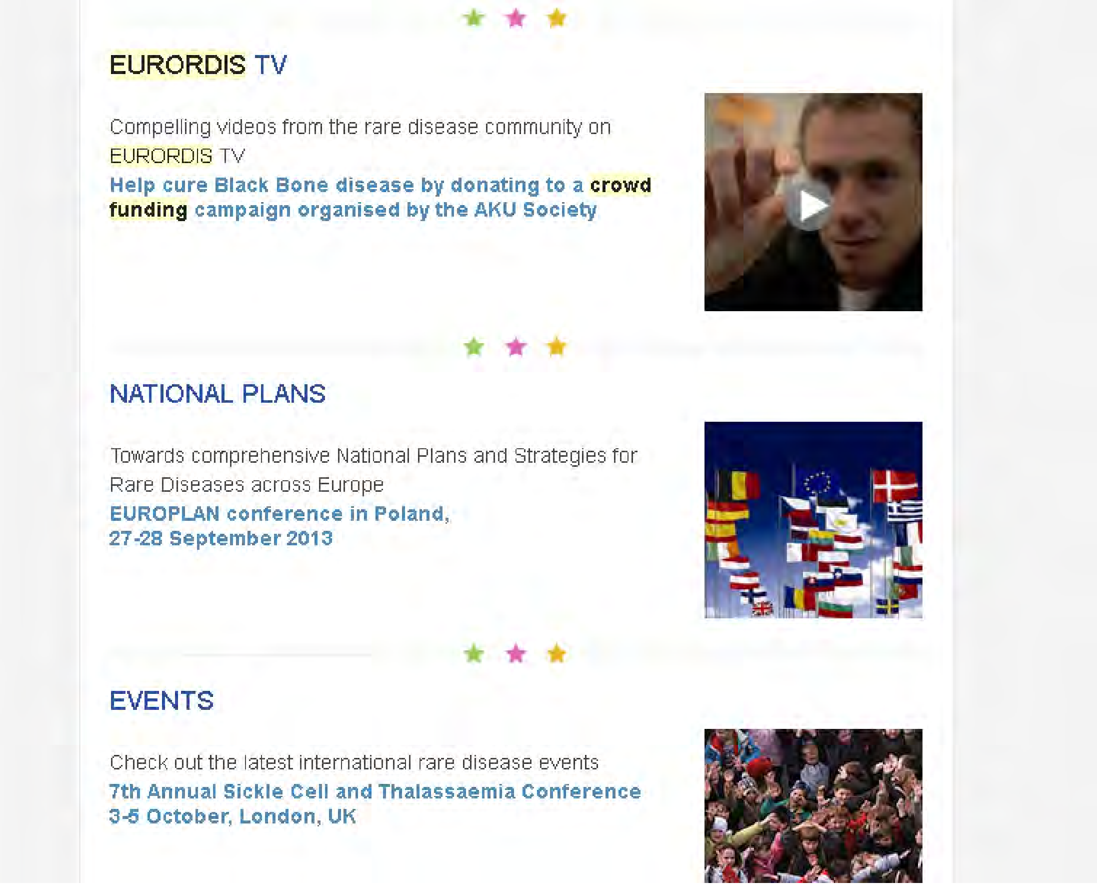
Eurordis networks



* * *

EURORDIS TV

Compelling videos from the rare disease community on EURORDIS TV

Help cure Black Bone disease by donating to a crowd funding campaign organised by the AKU Society

* * *

NATIONAL PLANS

Towards comprehensive National Plans and Strategies for Rare Diseases across Europe

EUROPLAN conference in Poland,
27-28 September 2013

* * *

EVENTS

Check out the latest international rare disease events

7th Annual Sickle Cell and Thalassaemia Conference
3-5 October, London, UK

We made it!

Our campaign had **28,598** visits over **50** days which led to **1469** donors from **40** countries donating a total of **$98,685** on Indiegogo.

$22,327 was also donated offline taking us to a grand total of **$121,012**

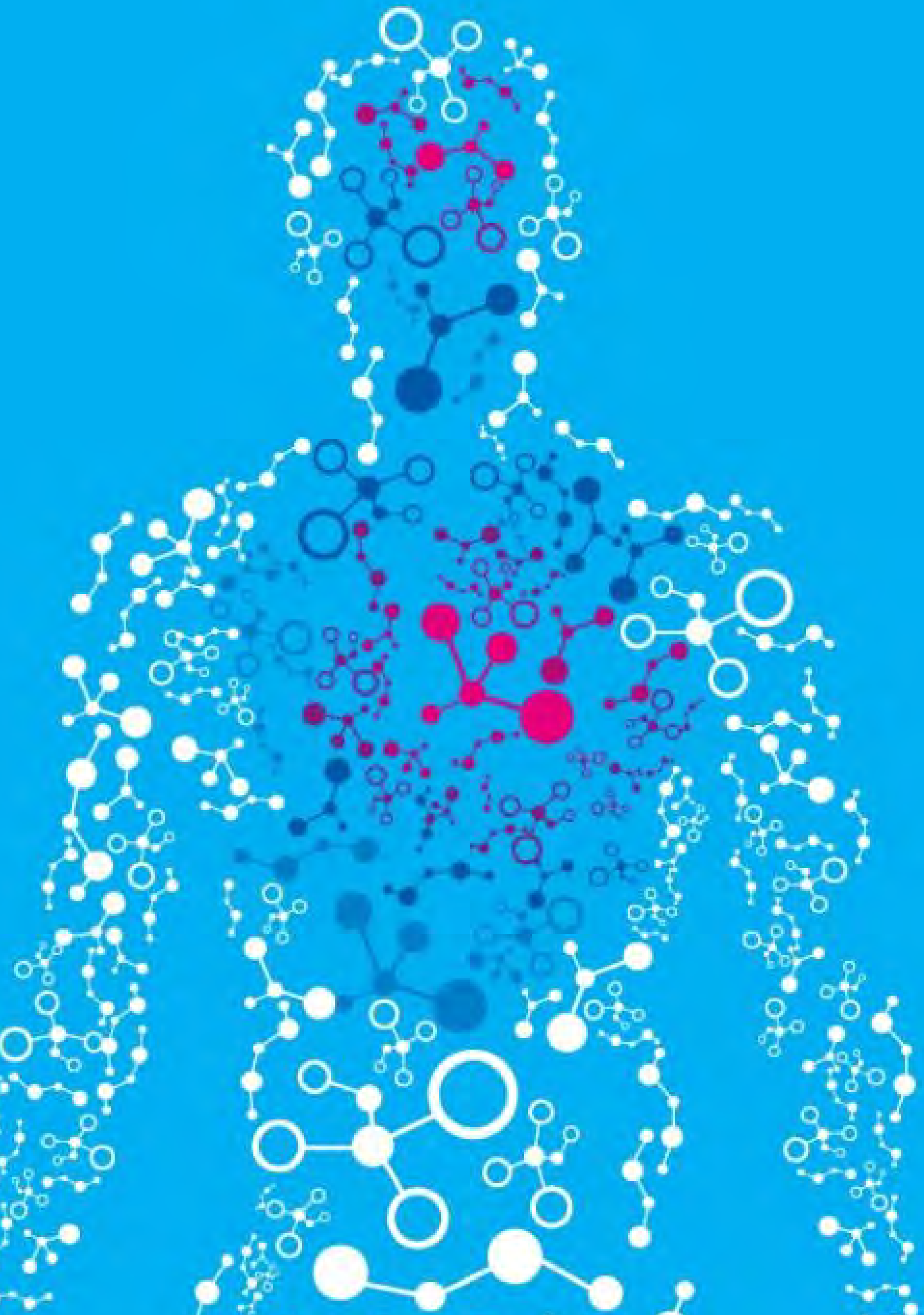
We couldn't have done it without your help. Every dollar donated and every email, tweet or facebook post sent has helped us reach our goal.

So we would like to say a great big

See our AKU campaign on Indiegogo

- http://www.indiegogo.com/projects/cure-black-bone-disease/x/1961731

Findacure: It’s time to care
Crowdfunding campaign



findacure

the fundamental diseases partnership

What is Findacure?

Findacure helps patients promote the search and development of treatments and cures for fundamental diseases

We do this by:

- Empowering patient groups to evolve into effective campaigners for change
- Facilitating patient groups to drive the development of treatments
- Campaigning for a receptive research environment

What are fundamental diseases?

Fundamental diseases are extreme and rare genetic disorders that offer a unique opportunity to better understand other diseases, including many common conditions.

Familial Hypercholesterolemia
↓
Heart disease

Hypophosphatasia
↓
Osteoporosis

Neuroacanthocytosis
↓
Parkinson’s and Huntington’s

Alkaptonuria
↓
Osteoarthritis

Congenital generalised lipodystrophy
↓
Type II diabetes

Fundamental Diseases: It's Time To Care

It's time to change the lives of those living with fundamental diseases.

Cambridge, United Kingdom   Health

Story   Gallery 7

Hi. We are Nick and Flóra, and together we make up Findacure: The

$0 USD

RAISED OF $25,000 GOAL

0%   ? time left

IN PARTNERSHIP WITH

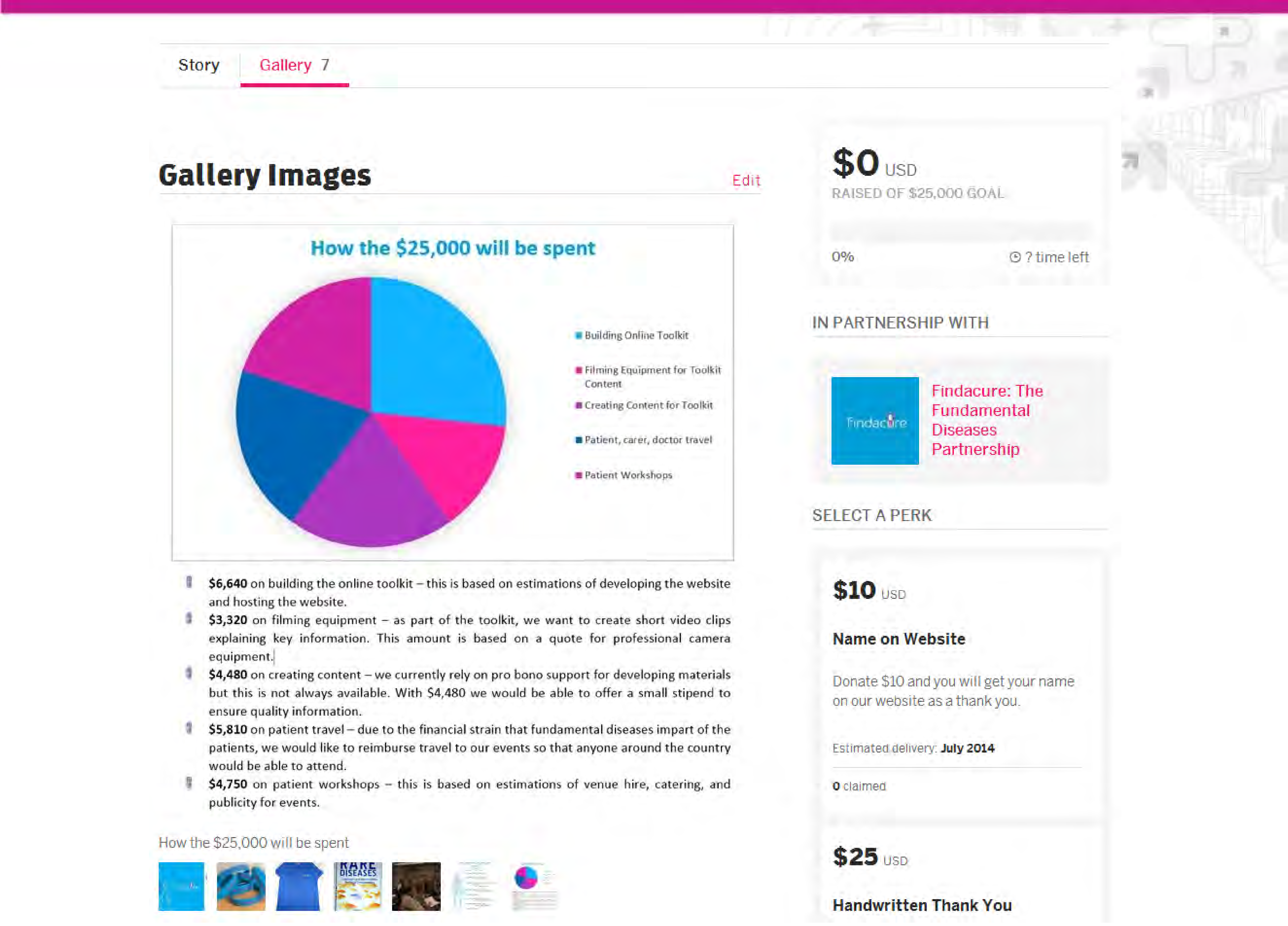
Findacure: The Fundamental Diseases Partnership

SELECT A PERK

$10 USD

Name on Website

Donate $10 and you will get your name on our website as a thank you.



Gallery Images

How the $25,000 will be spent

- **$6,640** on building the online toolkit – this is based on estimations of developing the website and hosting the website.
- **$3,320** on filming equipment – as part of the toolkit, we want to create short video clips explaining key information. This amount is based on a quote for professional camera equipment.
- **$4,480** on creating content – we currently rely on pro bono support for developing materials but this is not always available. With $4,480 we would be able to offer a small stipend to ensure quality information.
- **$5,810** on patient travel – due to the financial strain that fundamental diseases impart of the patients, we would like to reimburse travel to our events so that anyone around the country would be able to attend.
- **$4,750** on patient workshops – this is based on estimations of venue hire, catering, and publicity for events.

How the $25,000 will be spent

$0 USD

RAISED OF $25,000 GOAL

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IN PARTNERSHIP WITH

Findacure: The Fundamental Diseases Partnership

SELECT A PERK

$10 USD

Name on Website

Donate $10 and you will get your name on our website as a thank you.

Estimated delivery: **July 2014**

0 claimed

$25 USD

Handwritten Thank You

Front side

Reverse side


findacure

Please donate to our crowdfunding campaign supporting fundamental disease patients at:

igg.me/at/f-d-care

#time4fd

igg.me/at/f-d-care

#time4fd

Please support our Findacure campaign on Indiegogo

igg.me/at/f-d-care

Crowdfunding: lessons learnt

Dos and don’ts

- Prepare and plan carefully
- Contact friends/family/networks beforehand
- Make a strong, short video
- Make it personal, but back it with a big team
- 7-9 perks
- Always thank your donors
- Work with the Eurordis team to promote it

Dos and don’ts

- Choose your platform carefully
- Get at least 35% from friends/family initially
- Get media coverage
- Ask your patient supporters to promote it
- Use social media
- Email people directly
- Frequent updates and comments