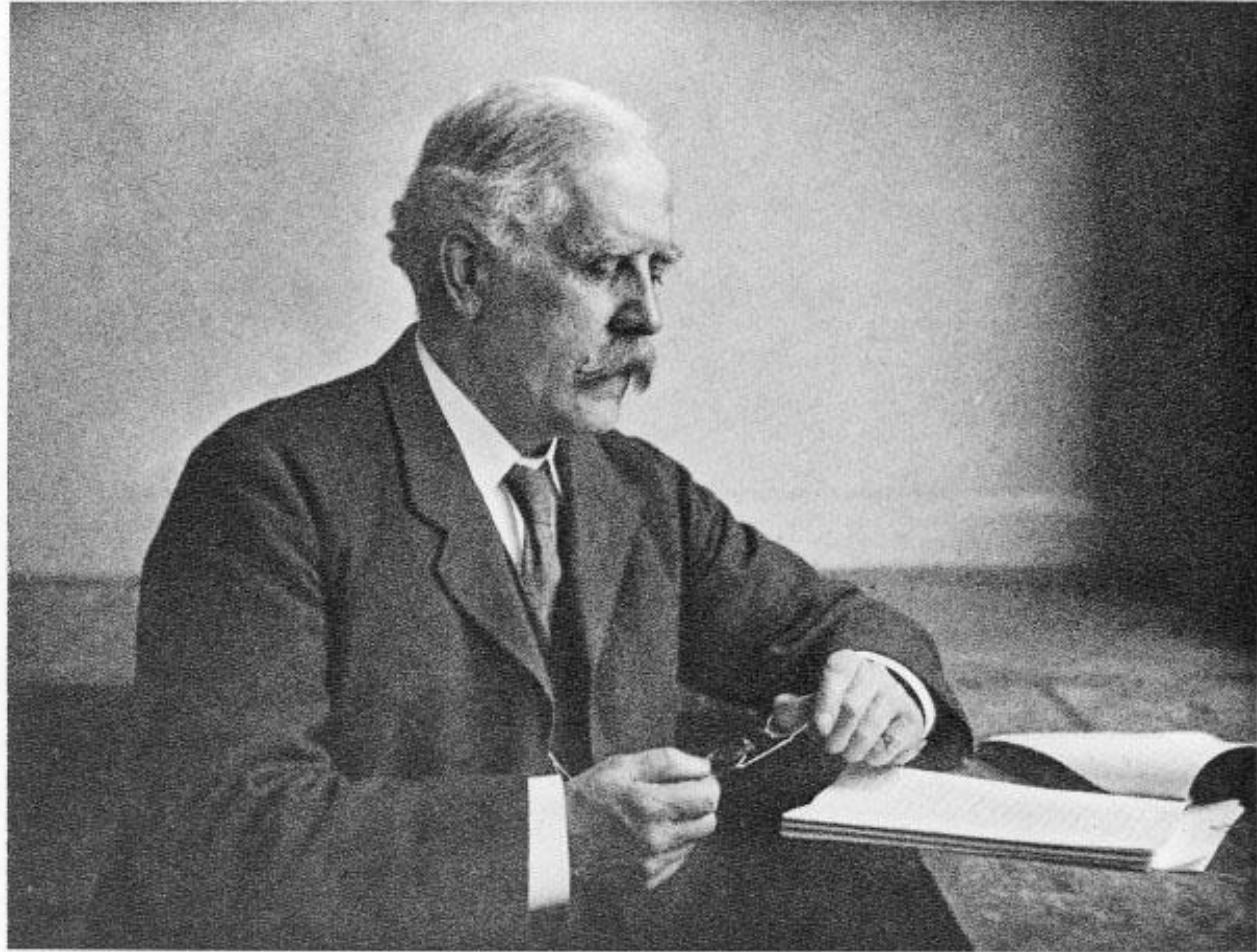


Fundraising for rare diseases: a case study from AKU

Dr Nicolas Sireau, Chairman and CEO, AKU Society

nick@akusociety.org

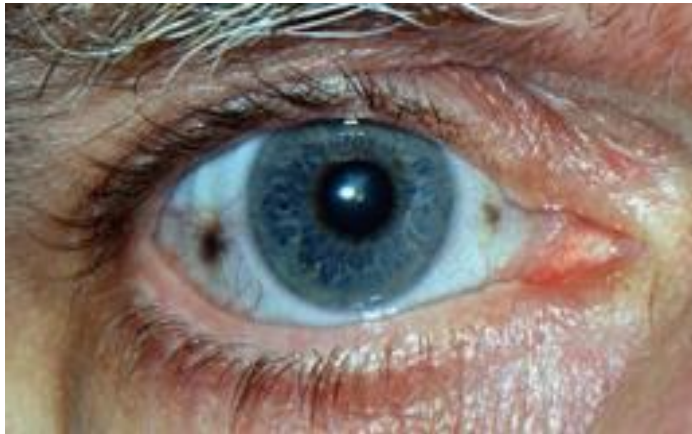
1902: Sir Archibald Garrod identifies Alkaptonuria (AKU)



A. G. Garrod,

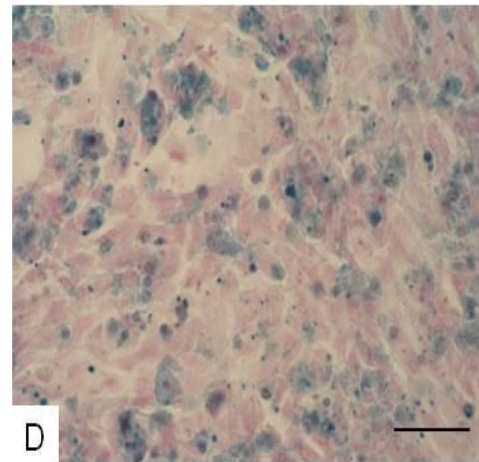
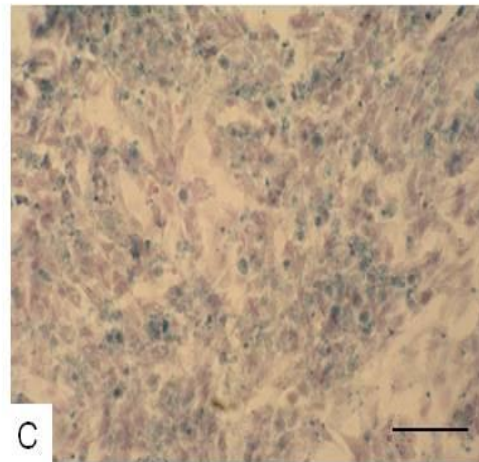
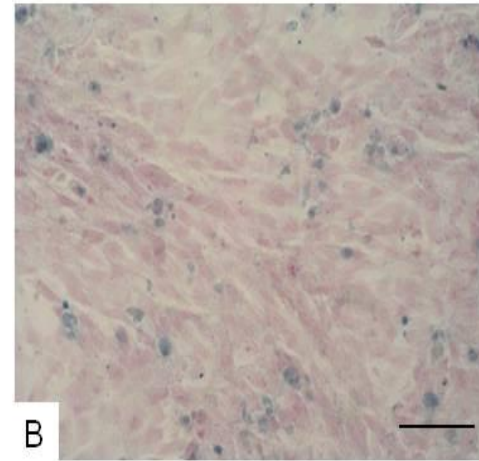
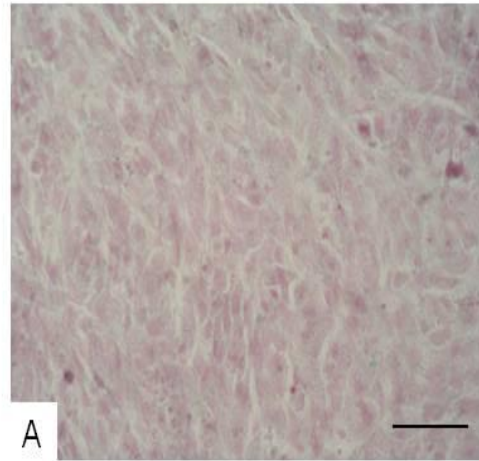
Step 1: fundraising for a post-
mortem
€5 000

The AKU tetrad



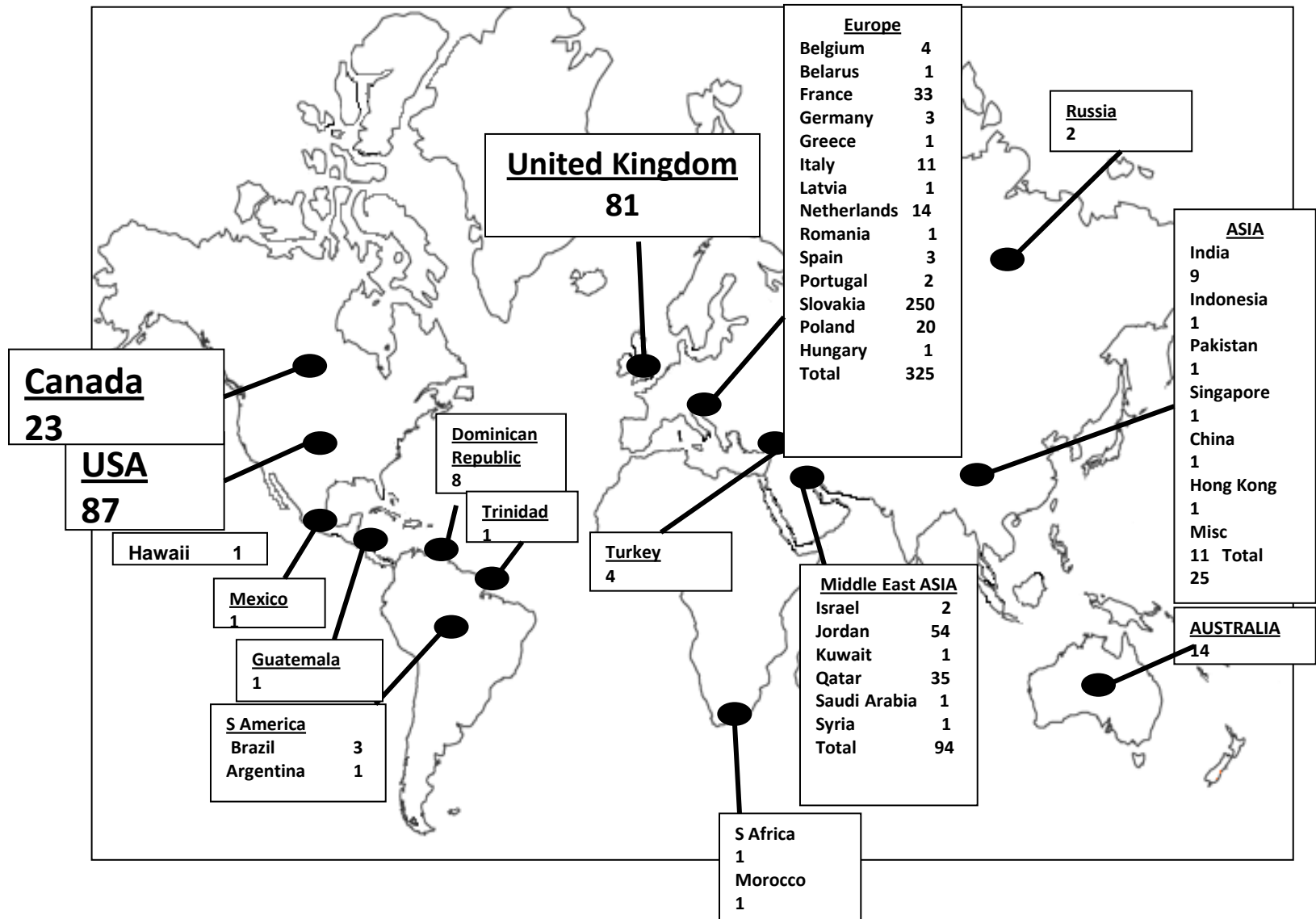
Step 2: fundraising for a cell model
€50 000

Developing a cell model



Step 3: fundraising for a patient
identification campaign
€250 000

A global patient movement



Step 4: fundraising for an animal
model
€600 000

AKU mouse model



Step 5: fundraising for a registry and
patient record system

€400 000

Welcome to PatientsLikeMe.

Male 38y
Alkaptonuria

This is your patient icon.
It summarizes your current status for other members.

Get 3 stars!

What else can you do?

- > [My Conditions](#)
- > [Bio](#)
- > [Picture](#)
- > [Quality of Life](#)
- > [My Symptoms](#)
- > [My Treatments](#)
- > [Weight](#)

Please fill out your **condition history**

Answer a few questions about your diagnosis status to make it easier to find other patients like you.

InstantMe

How are you feeling now? **Why?** (optional)

Very good

Good

Neutral

Bad

Very bad

You're signed up for **email reminders** to update your InstantMe

Text limit: 140 / 140 left

Patients Like Me



We found **1 patient** like you.



others to PatientsLikeMe

Updates + Feeds

You can choose to **subscribe** to



PATIENTS KNOW BEST®
MANAGE YOUR HEALTH

Sign in

Sign up!

Welcome Why Patients Know Best? Resources About us

Welcome to Patients Know Best



Read letter from our CEO



Step 6: fundraising for a National
AKU Centre and observational study
€6m



The Royal Liverpool and
Broadgreen University Hospitals
NHS Trust



Welcome to
**The Royal Liverpool
University Hospital**

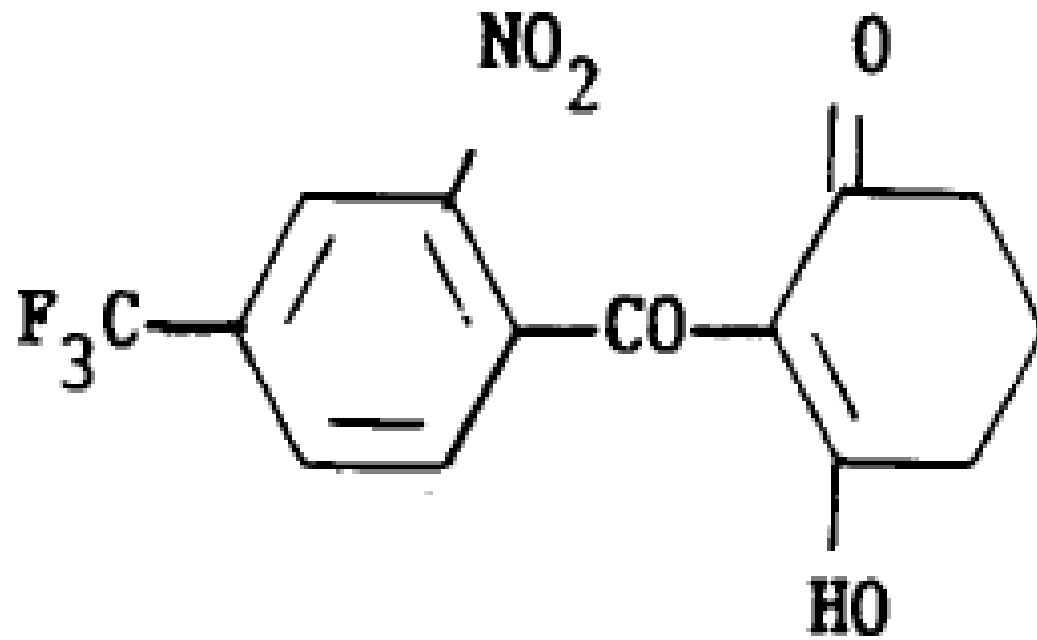
ROYAL LIVERPOOL

Average cost of an AKU patient

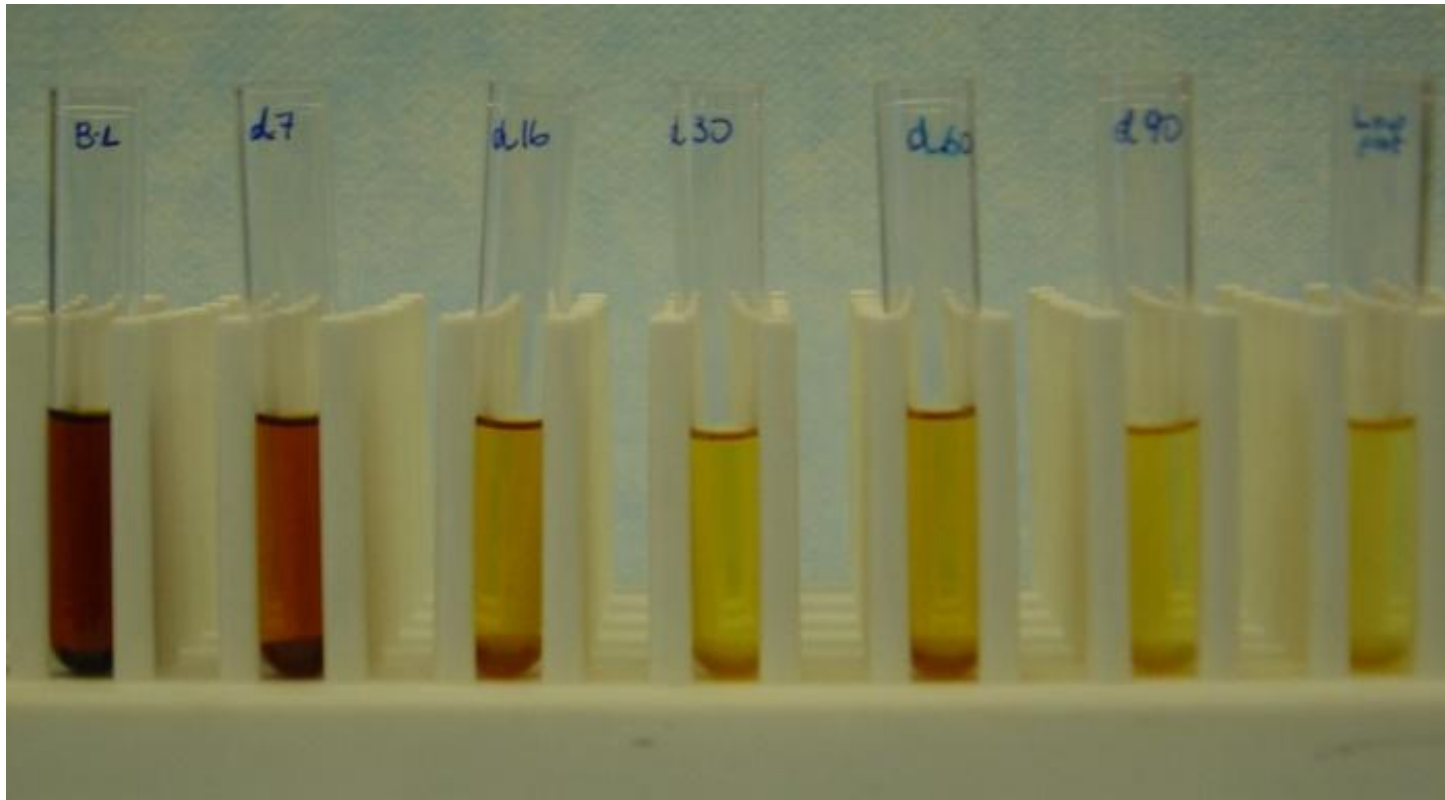
- Direct costs from one AKU patient for one year can be in excess of £100,000
- A weighted average of all scenarios shows total direct health care costs of approximately £1m may be a reasonable conservative estimate
- A conservative approximation of total costs of AKU in UK including indirect costs (lost wage and production) is £1.4m - £2m per year, with the upper limit as high as £7m

Step 7: fundraising for an
international clinical trial
€10m

Nitisinone



Nitisinone for AKU



National Institutes of Health

The partners

The Royal Liverpool and
Broadgreen University Hospitals
NHS Trust

NHS



UNIVERSITY OF
LIVERPOOL



Hôpital Necker
Enfants Malades

PSR
the orphan EXPERTS

UDOS
Drug development consultancy

nordicbioscience

UNIVERSITY OF SIENA



AKU
+ alcaptonuria society →

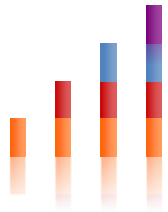
sobi
SWEDISH ORPHAN BIOVITRUM

ALCAP
Association pour la Lutte Contre l'Alcaptonurie

The different forms of fundraising

The challenges

The opportunities:
AKU as a fundamental
disease



findacure

the foundation for fundamental diseases

nick@akusociety.org