Fundraising for rare diseases: a case study from AKU

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1902: Sir Archibald Garrod identifies Alkaptonuria (AKU)
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

AKU Research Team, Liverpool
Step 3: fundraising for a patient identification campaign
€250 000
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.

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

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Welcome to Patients Know Best

Read letter from our CEO
Step 6: fundraising for a National AKU Centre and observational study €6m
Welcome to The Royal Liverpool University Hospital
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 cost 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 different forms of fundraising
The challenges
The opportunities: AKU as a fundamental disease
findacure
the foundation for fundamental diseases

nick@akusociety.org