

Ben Merrett

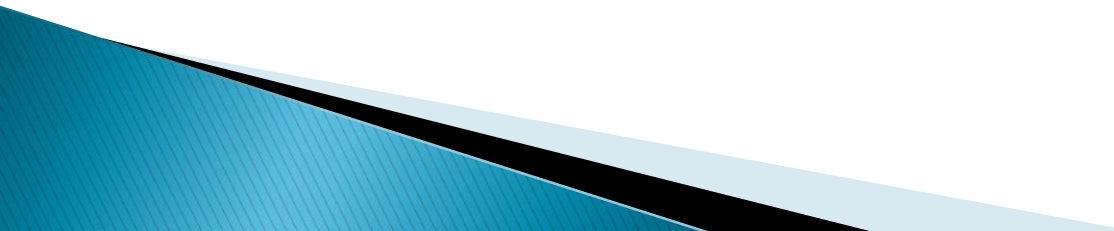


- ▶ Fundraising
- ▶ Collaboration
- ▶ “How can DEBRA groups work together with small amounts of funding and make big achievements?”

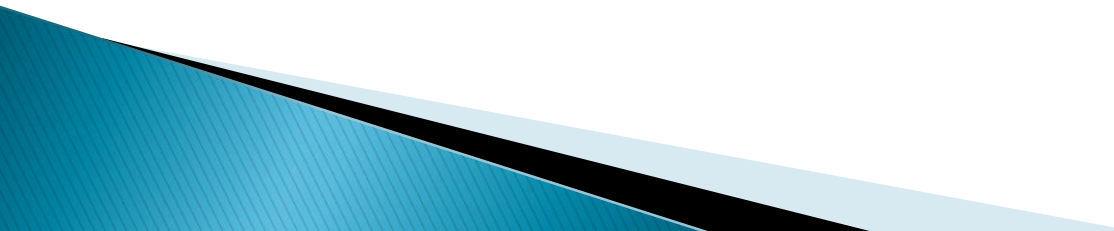
Phyllis Hilton



What is Fundraising?

- ▶ Raising awareness by telling true stories
 - Pain and disfigurement
 - ▶ Asking for help
 - It is a compliment
 - ▶ Thanking
 - Genuine Gratitude
 - ▶ Networking
 - Who else could help me?
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Fundraising for nothing

- ▶ Events underwritten by a sponsor
 - ▶ Create an attraction – Pull people in
 - ▶ CSR
 - ▶ Foundations, Trusts
 - ▶ Challenges – Run, Walk, Swim
 - ▶ Do something different
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- ▶ Volunteer inspired / Employment driven
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Successful Fundraising models

- ▶ Direct Mail – Austria
- ▶ Small network of affluent donors – SRF – \$3m
- ▶ Joesph family – \$5m
- ▶ Charity Retail – 4 DEBRAs now active
 - Large numbers of people giving small amounts
- ▶ High net worth individuals. Golf, shooting, gala events
 - Small number of people giving large amounts
- ▶ Cry and Inspire, entertain
- ▶ Images, Easy messaging, case for support
- ▶ Nothing more powerful than the EB voice

Sir Timothy John Berners-Lee OM KBE FRS FREng FRSA FBCS

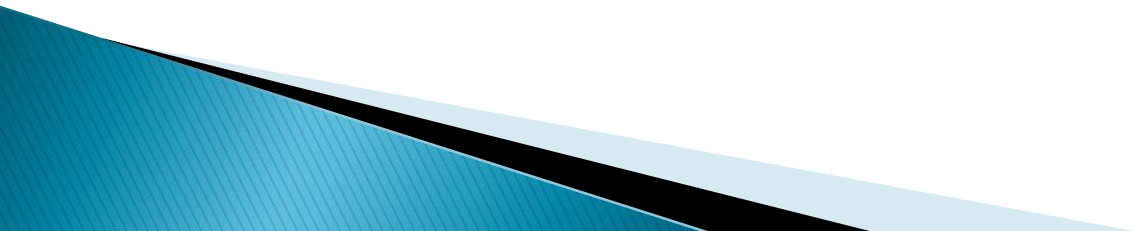


Are we at this stage yet?

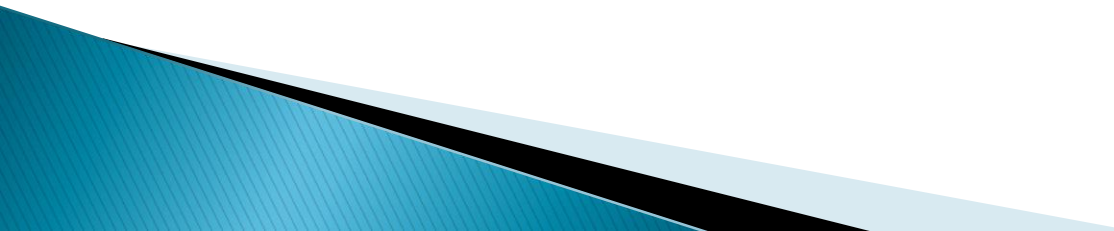




Why have we historically collaborated?

- ▶ Very lonely on your own.
 - ▶ Nonsense not to share information.
 - ▶ It is a global challenge. Invest in best Research world wide.
 - ▶ Current clinical understanding needs to be shared
 - ▶ Best practice understanding is applied everywhere
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Why will Big Pharma invest?

- ▶ EB affects the few, we are unknown, cost of research enormous.
 - ▶ BUT
 - ▶ Governments set aside big \$ for rare conditions
 - ▶ Orphan drugs easier to gain regulatory approval – extended exclusivity
 - ▶ As so severe hospital ethics approval for experimental trials is easier
 - ▶ Once they specialise they are likely to re-invest
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Big Pharma hunting for treatments /patients will gravitate to DI

- ▶ Amicus – Zorblisa – Topical cream –\$847m buyout. “Market worth \$1 billion dollars–plus”
- ▶ Abeona Therapeutics– Gene Therapy – “Phase II clinical trial in 3Q2016”
- ▶ Fibrocell Science Inc – Fibroblasts “enrolment in PI clinical trial Q22017”
- ▶ Repositioning drugs. Losartan , Rigersertib, Diacerein
- ▶ Shire – Acquired Lotus – intravenous protein replacement
- ▶ Amryt Pharma – Episalvan – “The global EB market is estimated to be worth approximately US\$1.5 billion per annum” “faster healing over 14 days of treatment for recent wounds and 28 days of treatment for chronic wounds” \$14m raised to fund a trial. Chairman is ex-founder of Shire
- ▶ World Biotech is USA centric; Patient enrolment becoming harder with over \$500m invested for EB

Collaboration

- ▶ Andrew Triggs Hodge. Triple Olympic Gold and quadruple world champion



Research Collaboration

- ▶ Regular DEBRA conferences
 - ▶ Sharing the specialist knowledge
 - ▶ Calls for papers done in partnership
 - ▶ Patient Panels
 - ▶ Keep asking our researchers for QOL improvement
 - ▶ Cystic Fibrosis Trust . \$40m invested. Sold for \$2.3billion
 - ▶ Linking our investments to Biotechs gives access to progress
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Colloboration !





Driving up standards – Registry

The Cystic Fibrosis Trust Registry is a secure collection of data about people with CF in the UK, collected with consent and used to improve care and to monitor new treatments.



10,583

people on the
UK CF Registry



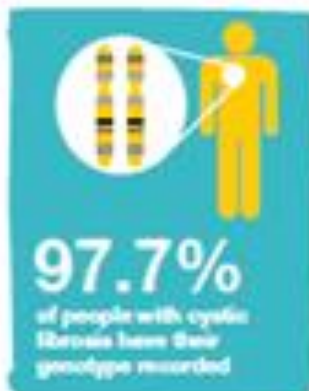
89%

have
complete
data



59.3%

of people with cystic
fibrosis are aged 16
or older



97.7%

of people with cystic
fibrosis have their
genotype recorded



72

transplants in 2014
compared with 57
the previous year



2 months

is the median age
of diagnosis



40.1

is the median predicted
age of survival

70.1%

of adults with cystic
fibrosis are in
employment or study

Small funding – Big Impact

- ▶ EB Registry
 - ▶ Best Practice papers
 - ▶ Conferences, EB–Clinet, International
 - ▶ Sharing Research understanding
 - ▶ Give Hope
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