Prioritising uncertainties assembled in UK DUETs, for additional research
James Lind Alliance

Tackling treatment uncertainties together

Patients and Clinicians

James Lind, 1716-1794
It's safe: John Gummer feeds his daughter Cordelia a burger
Consumer workshop on clinical trials for CJD

26 July 2002

Report of the meeting
Briton cured in CJD drug trial

by ANDREW CHAPMAN, Mail on Sunday

A British woman suffering from variant CJD has made an astonishing recovery after becoming the world's first human guinea pig in a pioneering drug trial.

Rachel Forber, a 26-year-old former soldier, was diagnosed with the disease in June, and given at most a year to live. As vCJD attacked her brain and nervous system, the fit and lively young woman lost the ability to walk, talk coherently, feed herself or even recognise her parents and surroundings.

Her father Stephen, who refused to believe Rachel was dying, spent long nights carrying out painstaking research on the Internet. He contacted Professor Stanley Prusiner, a Nobel Prize winner honoured for his work in identifying the prion - an infectious protein - as the cause of BSE in cattle, and CJD in humans.

And after receiving treatment from the team led by the professor in California, Miss Forber has undergone an extraordinary transformation.

She can now walk unaided, talk, eat with a knife and fork and perform co-ordination tests which before treatment were impossible.

News of Rachel Forber's progress will give hope to other sufferers of the deadly disease. There have been 106 victims of variant CJD since 1996, of whom seven are still alive, but Professor Prusiner's team insists it is too early to talk about a cure.

Details of the drug trial remain secret but the Department of Health confirmed that preliminary results, due to be made public this week in a scientific journal, are...
Teenager gets CJD treatment

A teenage patient dying from vCJD has finally received a controversial treatment which might slow the progress of the disease.

Jonathan Simms, 18, from Belfast, will now be given repeated infusions of the drug pentosan polysulphate directly into his brain.

The initial procedure is reported to have gone smoothly.

Jonathan's father, Don, and the family of another teenage vCJD patient took an NHS trust to court to get the treatment approved.

While laboratory experiments suggest it has the potential to tackle the condition, the drug is controversial because it has only received limited testing in animals.

"It's an experimental treatment but it's not for experiment's sake," Don Simms.
Life goes on in shadow of cruel disease

Published Date:
06 March 2009

By Sarah Freeman

When Holly Mills was diagnosed with variant CJD, she was given just months to live. Six years on Sarah Freeman reports on her ongoing battle.

Much has been written recently about the right to die. For the last six years the Mills family from North Yorkshire have been battling for their youngest child’s right to life.

Variant CJD victim Holly Mills, 23, at home with her parents Linda and Peter.
Family friend of John Gummer is killed by CJD aged 23

By ANDREW LEVY

Last updated at 19:16 11 October 2007

Elizabeth Smith: She learned on her 21st birthday that she had vCJD
Who are we?

Formed in 2004 by:
- Royal Society of Medicine – *Dr John Scadding*
- James Lind Library – *Sir Iain Chalmers*
- INVOLVE – *Sir Nick Partridge*

Funded by DH and MRC till 2013, *to see if it works*

Tackling treatment uncertainties - together (patients and clinicians)
Who are we?

We are a small team – 4 part timers!

Lester Firkins      Sally Crowe     Patricia Atkinson    Katherine Cowan
Support Priority Setting Partnerships

Support and raise the profile of patient / clinician involvement in priority setting

Gain evidence and share
The five stages of a PSP

- **Initiation**
  - Who starts it off

- **Consultation**
  - Who do we need to tell

- **Collation**
  - Collecting uncertainties

- **Prioritisation**
  - Sorting them into order

- **Reporting**
  - The funding opportunities.
• Priority Setting partnerships
  – Asthma
  – Urinary Incontinence
  – Prostate Cancer
  – Vitiligo
  – Life after Stroke in Scotland
  – ENT – Aspects of balance
  – Intensive Care Units (Scotland)
  – Schizophrenia
  – Epilepsy
  – Type 1 Diabetes
  – Type 2 Diabetes
  – Cleft Lip and Palate
  – Head and Neck Cancer
  – Eczema
  – Pressure Ulcers
  – Lyme Disease
Welcome to the James Lind Alliance Guidebook.

The Guidebook provides step-by-step guidance to establishing Priority Setting Partnerships. These bring patients, their carers and clinicians together to identify and prioritise treatment uncertainties for research, using James Lind Alliance (JLA) methods.

You can access the Guidebook and the JLA Protocol using the menu bar on the left.
WIIFM - researchers

- Questions which matter
- Ideas that make a real difference
- Support in the ethics process
- Participation within the research itself
- Advocates and disseminators

“Certainly I as a researcher will use this information to inform what projects we take forward in future.” UI PSP

“It has helped to clarify research uncertainties, and hopefully it will give incontinence research some credence and standing. Too often, funding is hard to come by because incontinence is considered not to be very sexy.” UI PSP
Seeking evidence

What's happening now?

• Clinicians are more involved than patients in the whole process (76.9% as opposed to 1.6%)
• Patients less likely to be consulted as to their research priorities than clinicians (18.2% as opposed to 88.5%)
• Clinicians and patients are more likely to work separately on identifying research topics, than collaboratively
• The James Lind Alliance Partnerships are highly distinctive

Stewart R, Oliver S. A Systematic map of studies of patients’ and clinicians’ research priorities, London James Lind Alliance, December 2008
Seeking evidence

What's happening now?

- The majority of research funding bodies (31 out of 52) operate in a responsive mode and **rely on researchers to submit ideas for research** and use a process of peer review to decide which applications to fund.

Staley K, Hanley B, (for the JLA) Scoping research priority setting (and the presence of PPI) with UK clinical research organisations and funders. Oxford December 2008
• There is considerable variation in the level of PPI in decision making process … some involve patients at every stage … others only involve lay members as Trustees at the very final stage.

• There is little evidence to suggest that generating a list of research priorities on its own has a major impact on the proposals developed by researchers.

Staley K, Hanley B, (for the JLA) Scoping research priority setting (and the presence of PPI) with UK clinical research organisations and funders. Oxford December 2008
• Professor Dame Sally Davies
• The NIHR aims to ensure that all our research projects have active public involvement right from the start.
• I have always taken the view that Public Involvement in research should be the rule, not the exception

• Forward to Exploring Impact INVOLVE 2009
Assessment of full applications:
Success rate 37%, large proportion significantly flawed.

1. Many proposals had inadequate arrangements (input and budgeting) for PPI in the design of the proposal.

Characteristics for a successful Programme Grant:

1. A proposal that clearly involves the public and patients in the development and delivery of the proposal by not only specifying the people dedicated to the role, but also allocating sufficient resource from within the budget to allow for this.

- Professor Adrian Grant
- Director, NIHR Programme Grants for Applied Research Programme
We are currently talking to people with an interest in vitiligo in order to establish the most important questions to be answered by research.

This work will focus on research into the treatment of vitiligo and we are keen to hear your views.

If you have vitiligo, are the parent of a child with vitiligo or a health professional with an interest in the condition, you can help us by completing this survey and telling us what questions you have.

The survey is very short and should take no more than 5 minutes to complete.

If you would like to take part in this study by completing the survey please click here to read the information sheet.
Then please click here to complete the survey online.

This independent research study is being supported through a programme grant funded by the National Institute for Health Research (NIHR). It is being coordinated by the Centre of Evidence Based Dermatology, University of Nottingham in collaboration with the James Lind Alliance.

If you would like further information or have any questions please contact us: by telephone: 0115 84 68633 or email: vitiligostudy@nottingham.ac.uk.
## Prostate Cancer priorities for treatment research – Initial full list

<table>
<thead>
<tr>
<th>Reference</th>
<th>Question</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Basic Biology</strong></td>
<td></td>
</tr>
<tr>
<td>001</td>
<td>Is prostate cancer caused by a virus?</td>
</tr>
<tr>
<td>002</td>
<td>Would a better understanding by patients of the natural history of prostate cancer improve outcomes?</td>
</tr>
<tr>
<td><strong>Prevention</strong></td>
<td></td>
</tr>
<tr>
<td>003</td>
<td>Are there effective prostate cancer prevention approaches?</td>
</tr>
<tr>
<td>004</td>
<td>Do vitamins have a beneficial effect in preventing prostate cancer?</td>
</tr>
<tr>
<td><strong>Risk Factors - Genetics</strong></td>
<td></td>
</tr>
<tr>
<td>005</td>
<td>Are genetic factors a good tool to predict prognosis of prostate cancer?</td>
</tr>
<tr>
<td>006</td>
<td>Can genetic testing for prostate cancer reduce the risk of being wrongly diagnosed, and over-or under-treated?</td>
</tr>
<tr>
<td>007</td>
<td>Does a family history of other types of cancer increase the risk of developing prostate cancer?</td>
</tr>
<tr>
<td>008</td>
<td>What is the evidence that dietary changes reduce the likelihood of recurrence of prostate cancer after radical treatment?</td>
</tr>
<tr>
<td>009</td>
<td>What direct effects of dietary fats, and particularly dairy products, help reduce the risk of, or provide a protective effect against, prostate cancer getting worse?</td>
</tr>
<tr>
<td><strong>Risk Factors - Diet</strong></td>
<td></td>
</tr>
<tr>
<td>010</td>
<td>Are there any dietary measures that can prevent prostate cancer or slow its progression?</td>
</tr>
<tr>
<td>011</td>
<td>Is there an increase in the number of younger men developing aggressive prostate cancer? If so, does it affect treatment outcomes?</td>
</tr>
<tr>
<td>012</td>
<td>What can I avoid to prevent the growth of a tumor in the prostate?</td>
</tr>
<tr>
<td>013</td>
<td>Would a “whole life” scoring tool predict the risk of developing prostate cancer and would using it allow me to make choices that would reduce my risk of developing prostate cancer?</td>
</tr>
<tr>
<td><strong>Risk Factors - Life-style and environmental</strong></td>
<td></td>
</tr>
<tr>
<td>014</td>
<td>Are there any geographical variations of prostate cancer incidence and treatment outcomes in the UK?</td>
</tr>
<tr>
<td>015</td>
<td>Does consuming alcohol affect my risk of developing prostate cancer?</td>
</tr>
<tr>
<td>016</td>
<td>Does excessive coffee drinking affect prostate cancer risk?</td>
</tr>
</tbody>
</table>
The James Lind Alliance: patients and clinicians should jointly identify priorities for clinical trials.

The formation of the Alliance could not be more timely.

The James Lind Alliance: tackling research mismatches.

...the coming years promise to be as exciting as the past ones.
Lind guidelines offer a checklist for research priorities

When it comes to medical treatment, the research community's agenda doesn't always match up with patients' desires. For instance, when people suffering from knee osteoarthritis were asked in 1999 what research was needed, they said they wanted more information on interventions such as braces, exercise regimens and pain management strategies. Yet most research at the time focused on drug and surgical treatments (Lancet 355, 2037–2040, 2000).

The Oxford-based James Lind Alliance was established in 2004 to help bridge such gaps. The nonprofit, funded by Britain's Department of Health and Medical Research Council, brings patients and clinicians together to identify top research priorities for a specific condition. On 11 May, the Alliance launched its online guidebook, intended to enable patients and clinicians to help set the medical research agenda (http://www.jlaguidebook.org/). The step-by-step manual explains how to establish partnerships among patient organizations and clinician groups, identify and prioritize unanswered questions in an area of science, and take the findings to funders.

"It strikes me as perverse that patients and clinicians aren't naturally the ones who determine priorities for medical research funding," says Lester Firkins, co-founder of the James Lind Alliance. "With the guidebook, we're giving people who want to try and set priorities an easy, structured way to do it."

"It's hard to imagine anyone in the world thinking this isn't a good idea," says Martin Burton, an ear, nose and throat (ENT) surgeon and president of the British Society for Academic Otolaryngology. The ENT community is forming a balance disorders partnership, the first of several disorders it plans to take on. "We're very interested in doing good, quality research for the benefit of patients, and the guidebook will be immensely helpful with that."

The do-it-yourself guide draws from the Alliance's experience working with patient and clinician organizations. In the past, for example, JLA helped walk the patient advocacy group Asthma UK and the research-oriented British Thoracic Society through the process of priority setting. Now, groups who want to form such partnerships without the JLA's direct assistance have the option of using the guidebook as they collaborate on priority setting. The guidebook, which took 18 months to write, includes questionnaire templates, draft agendas and case studies of different partnerships.

"Ideally, an uncertainty that wouldn't have come to light otherwise goes forward, is researched and makes a real difference to the patients and to the people who treat them," says Katherine Cowan, the guidebook author. "The guidebook is our online resource for how you do that."

"The guidebook is brilliant—I wish we'd had it a year and a half ago when we started the priority-setting process," says Emma Halls of the Prostate Cancer Research Foundation, which has gathered 163 research-oriented questions from clinicians and individuals with prostate cancer. Patients and clinicians will vote in September on the final ten questions, which will help guide the foundation's funding. The findings will also be entered in a national database to help direct future federally funded research. "As a charity completely funded by donors, it seemed really natural to ask clinicians and patients for their input. But it's really complicated," says Halls. "The James Lind Alliance has made it so much easier to manage the process."

Alisa Opar, New York

Correction

The article "Universal" immunizations get a boost in India' (Nat. Med. 16, 497, 2010), described the Indian Drugs and Pharmaceuticals Limited as "the largest vaccine producer of its kind." However, although the company is the country's largest public-sector producer of drugs and pharmaceuticals, it is not the largest vaccine producer. The text should have read "A 7.6 billion rupee ($170 million) revival package was, as Nature Medicine went to press, still awaiting a cabinet nod to perk up the almost 50-year-old, government-owned Indian Drugs and Pharmaceuticals Limited (IDPL), the country's largest public sector producer of drugs and pharmaceuticals." The error has been corrected in the HTML and PDF versions of the article.
REPUTATION

A reputation that took decades to build can be threatened by a single event.