

Consumer workshop on clinical trials for CJD

26 July 2002

Report of the meeting



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Aim

To enable consumer understanding of clinical trial design, and to provide an opportunity for consumers and researchers to share ideas on the design of trials for CJD.

Objectives

- 1 For consumers and some of the CJD trial investigators to get to know each other
- 2 For consumers to be able to describe a randomised controlled trial, understand its benefits and drawbacks, and to be aware of quality criteria needed to ensure that the results are robust and reliable
- 3 To enable the consumers to interpret clinical trials for CJD
- 4 For consumers to have discussed and recorded their issues and questions regarding CJD trials and the PRION-I trial as a first example

Attendees

The workshop was attended by 41 individuals; 20 consumer representatives, nine researchers and clinicians, five individuals from the Department of Health (including the Deputy Chief Medical Officer – Dr Pat Troop), seven from the Medical Research Council (including four in the organising team). The consumer representatives brought experience of all forms of CJD, some as carers and some as people at risk. The group included individuals and couples, some who already knew each other, but many who had not met before. A complete list of attendees can be found in Annex I.

In initial discussions, attendees highlighted the following expertise that they brought to the meeting:

- First hand experience of CJD and its impact on families
- “Man in the street” perspective
- Experience of involvement in the pilot study on quinacrine
- Caring for individuals with CJD in clinical environments
- Caring for relatives with CJD in domestic environments
- Long term care of individuals with dementia
- Holistic knowledge of different types of CJD
- Experience in recognising neurological symptoms and diagnosing CJD
- Persuading someone to be part of a trial in another disease area
- Knowledge of the development of trials in the early days of HIV/Aids
- Recruiting people to clinical trials
- Experience of being in a high risk category for CJD

The meeting

The programme for the meeting and details of the discussion are set out in Annexes 2, 3 and 4 and these are worth reading to obtain a full flavour of the day. The style of the event was interactive with people working in groups to develop their ideas. To enable free expression, it was agreed that reports of the discussion would be non-attributable. In essence the meeting was split into two sessions.

The morning was dedicated to facilitating understanding of clinical trials; why they are undertaken, their design, and what it means to be involved in a trial. Attendees had the opportunity to design a study to substantiate claims made in adverts for everyday products and soon appreciated at first hand the similarities between this and developing trials to test drug efficacy. The morning ended with a final session where attendees were asked to write down all the questions that they would wish to be addressed when designing a trial for CJD. For ease of presentation these have been grouped and can be found in Annex 3.

The afternoon focussed more specifically on designing trials for CJD. It commenced with some formal presentations, giving background as to how the UK came to be designing a trial for quinacrine, the difficulties in designing trials for CJD, the difficulties in living with the disease, and a presentation on the first proposed example of a UK trial for CJD in the form of PRION-1. Armed with this knowledge, breakout groups then proceeded to discuss the issues raised in the final session in the morning. For ease of working the questions were assigned to 4 key themes although it was noted that there was overlap. They were:

- Living with the trial
- Communication
- Consent and ethics
- Trial design

An overview of the issues that were discussed in the afternoon session can be found in Annex 4.

Summary of main issues

The following points are some of the key issues that resulted from discussion at the meeting. Further details can be found in Annex 4.

- There was strong support for a trial and establishing it as soon as possible.
- There was strong support for data being collected for research purposes provided it did not cause undue pain or undue stress.
- There was a wish for the design to be flexible to allow for the roll-out of new therapies, to allow patients to discuss with trialists (people doing the trials) the amount and type of follow up they would be willing to undertake and to allow more flexibility in entry criteria.
- At the present time randomisation with deferred treatment was unlikely to be popular and deferral for 24 weeks may not be practical if the majority of cases to be included in a trial were to be sporadic CJD cases.
- Attendance at a tertiary referral centre for a first assessment and follow-up appointment should where possible not require more than 2-3 hours travelling time. Where possible, follow up should be done locally or at home. Assessments that could be done by carers should be explored.
- Entry criteria to the trial should not require tonsil biopsy. It may be offered where evidence for vCJD is not substantial, but the advantages and disadvantages should be explained so that an informed decision could be made.

- Researchers recruiting patients to the trial would need to be excellent communicators and understand some of the specific issues for CJD families.
- Expenses should be covered for patients, carers and dependants to attend trial follow-ups. Patients and carers would welcome support in organising travel and accommodation when required.
- Results from tests should be made available as soon as possible, with appropriate advice and interpretation.
- There should be full involvement of carers in decision making for patients to enter the trial, for their care while on the trial and in the amount of ongoing follow up they will undertake for research purposes.
- Communication was considered very important particularly to ensure the trial was accessible to every possible participant. Clinicians must work together to ensure patients have speedy access.
- Numbers on the trial could perhaps be improved by broadening the study internationally.
- Familial CJD at risk cases may be willing to be randomised in a trial in preclinical stages provided they were not notified of their genetic status and those who were not at risk were allocated placebo.
- DH and MRC must ensure good publicity for the trial and where clinical services are available.
- Good information should be available to support patients and carers to highlight scientific developments and thus alert them to new potential therapies. This supportive information would allow informed choices. A monthly newsletter was considered as one possible way of keeping the community informed.

Post-meeting feedback

Attendees of the meeting were asked to complete a questionnaire, which sought views on the value of the workshop. A report collating the responses has been written and can be found in Annex 5. The feedback may best be summarised by two quotations:

"everyone at a high level is really thinking about the issues"

*"a rare occasion where consumers/families **really** feel that they have been listened to".*

Outcomes

It was agreed that the report of the day would be used to inform the next stages of clinical trial development for CJD and a copy would be sent to the Chief Medical Officer.

Attendees of the meeting were invited to remain on a mailing list to enable further consultation as the field developed.

MRC would use the evaluation of this event to help in achieving greater consumer involvement in the activities of the MRC in other disease areas.

Acknowledgements

The MRC would like to acknowledge the input made by all those that attended. In particular MRC would like to thank:

- Carers, families and patient support groups for CJD.
- The Chairman for the day, Professor Ray Fitzpatrick, University of Oxford
- The Facilitator Mrs Sally Crowe, Training and Development Consultant (Crowe Associates)
- The Speakers:
 - Dr Pat Troop – Deputy Chief Medical Officer
 - Dr Sarah Walker – Statistician, MRC Clinical Trials Unit
 - Professor Janet Darbyshire – Director, MRC Clinical Trials Unit
 - Mr Lester Firkin - Honorary Chairman, Human BSE Foundation
 - Professor John Collinge – Director, MRC Prion Unit and Director, National Prion Clinic
 - The Department of Health for financial contribution to the meeting
 - The Treasurer of the Royal College of Physicians for kindly making available the venue.

Attendee list

Professor Ray Fitzpatrick
Chairman

Dr Robert Bennett
Medical Research Council

Mr Arthur Beyless
CJD Support Network

Mr David Body
Solicitor

Ms Sarah Buckland
Consumers in NHS
Research

Professor John Collinge
MRC Prion Unit, National
Prion Clinic

Mrs Sally Crowe
Training & Developmental
Consultant

**Professor Janet
Darbyshire**
MRC Clinical Trials Unit

Mrs Janice Emery
Carer

Dr Karen Finney
Medical Research Council

Mr Lester Firkins
Human BSE Foundation

Mrs Wendy Firkins
Human BSE Foundation

Mrs Janet Gibbs
Carer

Mr Peter Gibbs
Carer

Mrs Frances Hall
Human BSE Foundation

Dr Mary Holt
Department of Health

Miss Amanda Horne
Medical Research Council (s)

Dr Rowena Jecock
Department of Health

Dr Emma Jones
Researcher, St. Thomas' Hospital

Dr Angus Kennedy
CJD Support Network, National
Prion Clinic

Dr Richard Knight
National CJD Surveillance Unit

Ms Margaret Leitch
National CJD Surveillance Unit

Dr Joe McNamara
Medical Research Council

Ms Elizabeth Mitchell
Medical Research Council (s)

Ms Claire Morris
National Prion Clinic

Mrs Shirley Nurock
MRC Consumer Liaison Group
and Alzheimer's Society

Mr Ian Parsons
Department of Health

Dr Mark Pitman
Medical Research Council (s)

Ms Francesca Probert
Carer

Ms Helen Savva
Carer

Mrs Sarah Shadbolt
CJD Support Network

Mrs Pene Sinnott
Carer

Miss Rahana Siraj
Medical Research Council (s)

Dr John Stephenson
Department of Health

Mr Matthew Sydes
MRC Clinical Trials Unit

Dr Dafydd Thomas
National Prion Clinic

Mr Roger Tomkins
CJD Support Network

Dr Pat Troop
Deputy Chief Medical Officer,
Department of Health

Mrs Gillian Turner
CJD Support Group

Dr Sarah Walker
MRC Clinical Trials Unit

Mr John Williams
CJD Support Network

(s) - secretariat

Programme

Objectives for the workshop

- 1 For consumers and some of the CJD trial investigators to get to know each other
- 2 For consumers to be able to describe a RCT, and understand its benefits and drawbacks and be aware of the quality criteria needed to ensure that the results were robust and reliable
- 3 To enable the consumers to interpret clinical trials for CJD
- 4 For consumers to have discussed and recorded their issues and questions regarding CJD trials and the PRIONI trial as a first example

- 9:30am **Refreshments and networking**
- 9:45am **Welcome and Introduction by the chair of the workshop**
Professor Ray Fitzpatrick, Chairman
- 9:50am **Introductions exercise - how are we going to work today?**
Sally Crowe, Training and Development Consultant
- 10:20am **What is a clinical trial?**
Exercise to establish the concept of a clinical trial followed by discussion on the quality criteria
Sally Crowe (*Handouts will be available*)
- 11:15am Coffee/tea break**
- 11:30am **Why do we need trials?**
Dr Sarah Walker, MRC Clinical Trials Unit
- 11:50am **What does it mean to be involved in a trial?**
Janet Darbyshire
- 12:10pm **Developing questions regarding CJD Trials**
Sally Crowe
- 12:30pm Lunch**
- 13:30pm **Reconvene meeting, brief welcome to new arrivals and introductions. Objectives of the afternoon session and review of activities in the morning session**
Professor Ray Fitzpatrick, Chairman (*Handout of themes from morning session*)
- 13:45pm **Introduction to the afternoon session and discussion**
Dr Pat Troop, Deputy Chief Medical Officer, Department of Health
(*Handout on Therapy Advisory Group*)

- 14:15pm **Difficulties in designing a trial for CJD**
Professor Janet Darbyshire, MRC Clinical Trials Unit
(Visual handout of trial and summary)
- 14:30pm **Living with the disease – the patient/carer perspective**
Lester Firkin, Human BSE Foundation
- 14:45pm Coffee/tea break**
- 15:00pm **The draft PRION-1 Trial Protocol - explanation of design and discussion**
Professor John Collinge, MRC Prion Unit and National Prion Clinic
- 15:30pm **Facilitated workshops discussing the issues identified in the morning session**
- 16:15pm **Plenary discussion and concluding remarks**
Professor Ray Fitzpatrick
- 16:45 Workshop finishes**

Event held at the Royal College of Physicians by kind permission of the treasurer.

Questions raised by attendees that they felt would need to be addressed when designing a trial for CJD.

Trial design

- What help will I get? – (logistics/transport costs)
- Carers committing patients to intervention for science not patient
- Period of time for trial
- Can it be blinded?
- What are you going to 'do' to people?
- What stage do you give up?
- How long would people be treated for?
- Can my son/daughter be followed up locally?
- What investigations do I have to have?
- What professionals would be involved/told?
- If you are on a trial can you move?
- Measurement of carers perception of improvement or success
- How will you know it's working?
- Do you wish to keep somebody alive in severe disability and quality of life impaired?
- Is there any evidence that the drug works?
- What if prolonged suffering is the only outcome?
- What is the likely benefit of the treatment?
- What is for the benefit of the patient and what is for science?
- Is the objective to make patient better – fullstop, or an attempt to improve quality of life?
- Will we be told afterwards if we had treatment or placebo?
- How will it help me? How will it help others?
- Financial help for costs for taking part – eg travel?
- Frequency of monitoring progress?

Care in trial – living with the trial

- Will I feel like a lab rat?
- I want quality not quantity for my son/daughter
- How will joining the trial affect my relative's care?
- Where do I have to go and how often?
- Practical issues about visiting Centre? – especially with sporadic cases
- Does entering the trial effect management?
- Will the trials interfere with everyday life: work, sport, participation, looks etc?
- Are any potential side effects reversible?
- How would you react if I said "But I know it is doing my relatives good" parent/child instinct for instance
- Are there any known side effects & do these outweigh the advantages?
- Will we be told about known side effects before we start the trial?
- How long will I have to stay in hospital? Or attend clinic?
- How will I know the treatment is working/not?

- How is benefit assessed?
- Who determines quality of life?

Consent

- Who gives consent?
- How do you consent someone who is confused?
- Question of consent if patient doesn't have capacity? Who gives consent? Taking responsibility on someone else's behalf.
- How will you get consent from my family member?
- Do I have a free choice?

Ethics

- Is it right to give placebo if we know that outcome will be 100% death?
- Is placebo ethical for CJD trial?
- How do I find out that the MRC trialists are honest about their claims?
- Can you ethically exclude a CJD patient from trying a new drug?
- Should clinicians be able to give only one intervention?

Communication

- Are you collaborating with other researchers looking at the same things?
- Will we be kept informed of disease progress (during trial)?
- Will you publish negative results?
- How will we raise awareness of the trial?
- I have CJD – would I be informed that there is a trial?
- Open & quick swapping of information? Improve patient management
- Is complete confidentiality in the patient's interest in this area of research?
- How do you communicate progress to me?
- How will you let me know the results?
- How are things so far? ie what degree of success?
- Are you entering this for you the carer or for your affected relative?
- Can we be sure doctors will share information on individual progress?
- What happens if the money runs out?
- What are the communication channels to consultants and clinical support?
- Will it be confidential?
- How will you ensure confidentiality? (ie media intrusion)
- How do patients find out whether the trial will happen?

Entry criteria

- Does going into trial affect diagnosis? (eg tonsil biopsy is prerequisite)
- Complication of other unrelated disease requiring other drug treatment?
- Can pre-symptomatic patients enter the trial?
- What is evidence to indicate trial is needed?
- Are you sure my child has vCJD?
- All types of CJD?
- Could genetic positive people be entered into the trial without knowing the result of their genetic test?
- How can I get on it?
- What if your clinician doesn't want to put you forward for the trial?
- How do I get the patient out of the trial?
- Is it available to everyone?

- How do I get selected for a trial?
- What happens if I stop?
- Are very sick people excluded because they can't travel?
- How do you decide who gets which?
- How soon can I get on the trial?
- What will it cost me? (Psychologically/Time)
- Who is supporting it? (sponsoring/monitoring)
- Will it hurt?
- Timescale of setting up trial?
- How do you do a trial with such small numbers?
- Length of trial?
- Method of administration? eg nasty taste, oral/local injection
- Can we take part locally?

Other issues

- What else is available?
- How is the trial being funded and for how long?
- What degree of variation in approach is given? (in view of different ethnic/minority group)
- How many other trials will I be asked to be on?
- Can I switch to something else if I want to? (ie another trial drug)
- Is my son/daughter receiving the front runner treatment?
- Are there similar trials occurring elsewhere in the world?
- How do I know this is the right trial compared to others?
- Can one swap 'trials' if something better comes up?
- Does being on the trial exclude us from any other new treatment that comes along?

Report of the afternoon discussions of the consumer workshop on clinical trials for CJD

It was noted at the end of the morning session that it would not be possible to discuss the large number of questions raised. It was agreed that four main recurring themes would be identified and where possible all questions would be accordingly assigned. The themes identified were:

- Living with the trial
- Communication
- Consent and ethics
- Trial design

There were some questions that formed a fifth miscellaneous category (see Annex 3). It was noted that these tended to be quite specific questions and could be easily addressed in the preparation of the final draft of the proposed protocol.

It was clear that several of the issues overlapped the theme headings and thus may have been discussed in more than one of the breakout groups. The points listed in the discussion sections below are not in any priority order and they have been reported without significant interpretation and minimal editing.

I. Living with the trial

There were five topics in this theme and these are detailed below:

Practical issues that effect the patient

- How far (travelling), how often – trial observation measurements?
- Financial cost of travel?
- Stay in hospital?

Side effects of taking the drug

- How much?
- What do we know already?
- Are they reversible?

Impact on family who are involved in caring for the patient

- Will it effect relatives' care?

Impact on current management of disease

- How will the trial be integrated with the care the patient will receive?

Outcomes

- How will you/I know if treatment is working?
- Who determines what is quality of life?
- What if prolonging suffering is the only outcome?
- Benefit for patient? Or science?
- How will it help others and me?

Results of discussion

- The amount and frequency of follow up for the trial should be agreed between the patient and physician where possible or between the carer and the physician.
- Care and management of the patient was considered to be different (and possibly better) if patients were enrolled in the trial. The 'Placebo Effect' and the psychological benefit to be gained from close monitoring and support were all seen as positive advantages to being enrolled.
- If no further stress was going to be placed on the patient, there was general support for enrolling in the trial and undergoing further assessments for the benefit of science.
- 2-3 hours was considered to be the maximum travelling time to get to a tertiary referral centre for the first assessment on entering the trial. This may also require provision of transport such as an ambulance for the comfort and ease of travelling of the patient.
- The length of stay of the first assessment should be kept to a minimum.
- Travelling was difficult for carers who may still need to work or who may have other dependants. Follow-up assessments for the trial (after the first assessment) could be done monthly, but should be done locally. One subsequent central follow-up visit might be acceptable.
- Some patients do not like being in hospital and allowance should be made for home visits if this is mutually agreeable. More use could perhaps be made of carers to repeatedly assess patients, if for example, structured forms were readily available.
- Repeated taking of blood samples was considered reasonable, but any procedure (unless solely for care), which required a general anaesthetic or a weeks stay (or greater) in hospital was considered to be too stressful for the patient and their family.
- Expenses for the patient to be involved in the trial should be covered as should those of the carer who will accompany the patient. This could also include dependants. Assistance with travelling arrangements would be helpful.
- There was protracted discussion about whether the fact that a CJD patient carried a donor card made the decision to include them in a trial easier; and further to this, when they died, whether it was easier to make the decision to offer their organs for research. While some felt it was easier, others found it difficult to agree even though they believed it was probably would be the patient's wishes and the research was important. Sensitive case by case handling was clearly needed.
- The issue of side effects and the latest clinical point at which someone could be enrolled in the trial prompted protracted discussion on quality of life: who decides on quality of life and what are the criteria to measure it. Provided no pain would be caused, there was always hope and it was concluded that specific criteria for having access to the drug should not be set. However, neither did anyone wish to prolong life if quality was poor. Carers wished to be involved in discussions with clinicians on these issues.
- If undergoing tests, patients and carers wish to know the results as soon as possible and not have to wait several months for batches of samples to be complete. If a small delay is unavoidable there should be a clear indication as to when the results would be available.

2. Communication

Between

- Trial team and patients/carers
- Trial team (MRC) and the wider world
- Trial team and other health professionals involved in the care of the patient

About

- Confidentiality
- Media scrutiny
- Understanding disease progression and effect (or not) of treatment
- Raising awareness of trial

Results of discussion

Managing expectations

- It was clear that media coverage of a potential therapy could raise expectations or conversely an adverse effect report could cause concern. It would be helpful if investigators would provide comments on other media stories for patients so they could have a professional view on the implications of the latest news.
- Trial centre to communicate direct with patients where possible to avoid delays.
- Every effort should be made to make sure every possible participant knows about the trial (in particular, there was some concern expressed that ethnic minority groups, for example were not coming forward). This could be done by:
 - Arranging media publicity accessible to all cultural backgrounds
 - Reciprocal notification between clinicians
 - The issue of making CJD a notifiable disease was raised, but it was noted that this may create more negative rather than positive outcomes.
 - Familial cases would need to be handled with special sensitivity.
 - Broadening the study overseas.
- Earlier diagnosis is part of the communication needs
 - Better, earlier assessment, ensuring a mechanism is established to speed up referral.
 - (Problem is that early symptoms of CJD are shared with a number of other illnesses).
 - Making it known what tests are available (eg Tissue biopsy, gene test, etc).
- Clinicians use patient confidentiality as an excuse not to communicate with each other. This should not be allowed to continue.
- Patients would like feedback during the trial.
- Data Monitoring and Ethics Committee and the Trial Steering Committee to tackle issues of interim results and how to communicate them with participants without compromising trial.
- Many modes of communication could be used (monthly newsletters were suggested as a good way of keeping all parties involved of trial news and developments).

Practical communication strategy needs

- Awareness of the trial could be raised amongst GPs, social services, neurologists, psychiatrists, etc Information to primary and secondary care may need to be different.
- Feed into general information on ongoing trials.
- Clear message is needed from DH/MRC regarding the trial and where clinical services are available.
- Good information to neurologists/ psychiatrists is needed to ensure early referral to trial at time of diagnosis.
- General awareness campaign – prion disease
- Get to every point of entry for information eg NHS Direct (DH already working on this).

3. Consent & ethics

Consent

- Who gives it?
- What about confused patients? Capacity of patient?
- Freedom of choice
- Role of family member in consent

Ethics

- Is it right to give placebo if outcome is 100% death?
- Integrity of MRC trial team
- Exclusion of CJD patient from trying new drug. Is it ethical?

Results of discussion

- There are good GMC and MRC guidelines on obtaining consent and these could be used in CJD trial literature.
- Consent can be given by:
 - Patient
 - Parent or legal guardian – child below 18 - child's consent should be sought where possible
 - Patient's clinician
- Consent issue could be difficult if family members have different ideas regarding entry of their loved one into the trial. Next of kin cannot give consent on a patient's behalf, although it is generally considered good practise that their agreement (or assent) is sought. The patient's clinician is generally deemed to be in a good position to weigh up whether it is or is not in the patient's interest to participate in a study and (if different to the patient's clinician) the 'assent' of the patient's GP should also be sought.
- Balance of the possible positives and negatives of the trial need explicitly explaining.
- Freedom of choice – Randomisation may not be viable at current time as the majority of patients would probably wish to know what they are getting.
- Carers making decisions for patients unable to give consent themselves may be more cautious than if the patient was making the decision for himself or herself.
- Patients being monitored in the trial may change their mind if a subsequent treatment were to become available. Could there be flexibility in the trial design to accommodate this?
- Clear information, timing and use what's already there.
- Why 24 weeks deferral in the randomisation and not 12? A lot of the cases entered in the trial will be sporadic CJD patients whose average clinical course to death is four months.
- Envisaged that different types of CJD may wish to go into different parts of the trial. Familial cases may be willing to be included, but might not wish to know their genetic status. They asked whether it would be possible to allocate placebo to those that were negative and drug to those that were positive without telling them.
- Skill of trial recruiter – understanding the CJD family issues (unbiased).
- New drug – monitoring.
- Integrity of the Trial team – the group discussed the various groups that would be overseeing the trial eg the Data Monitoring and Ethics Committee.
- Ethics committee – the constraints and the advantages of being in a trial.
- A general wish that the trial with quinacrine should be started as soon as possible.

4. Trial design

Entry criteria

- **Who** can join?
- **How** do I get selected?
- 'sick' people too ill to travel
- evidence to indicate trial is needed?
- Can **pre-symptomatic** patients enter?

Trial design

- Can it be **blinded**?
- **Length** of trial?
- **Local** follow up?
- What are the **investigations** I will have to have?
- Who is **sponsoring**?

Results of discussion

It was felt that several of the issues overlapped and the following points were made. These are not in any priority order:

- Where patients were unable to communicate their wishes – there should be discussion between the carer and the physician.
- There were concerns over exclusion criteria – someone near the end of life. The decision to have access to a drug should result from discussion between the next of kin (often the carer) and the physician.
- Entry criteria does not need tonsil biopsy.
- Evidence for specific drugs – important to make this information available.
- Presymptomatic individuals eg individuals at risk of familial CJD might not wish to be included in the trial. The risk of side effects may be considered to be too great at the present time in what appear otherwise healthy individuals.
- Noted that blinding of the drug quinacrine is difficult.
- Intensity of monitoring agreed for all - intensity has to be on case by case basis.
- At home assessment important and should be accommodated as much as possible.
- Advantages and disadvantages of a specialist centre were noted – care issues at such a centre compared with locally would be different.
- The importance of testing in a controlled way for consistency was noted.
- Care will be optimised in the trial – benefit that patient not as lonely and there would be good back up support.

Summary of evaluation for the consumer workshop to discuss clinical trials for CJD

This summary is derived from eight completed evaluation forms and four e-mail correspondence received from attendees. This represents approx 25% of the total workshop participant population, although some participants were not able to attend all of the sessions.

This summary is in the form of themes derived from the written feedback, and reflections of the workshop team, on and after the day.

Overall the workshop seemed to achieve its stated objectives and in a manner that was inclusive, relaxed and respected the different experiences and perspectives, that people who attended, had of CJD. The effort of the workshop team to create a welcoming and productive atmosphere, with a balanced programme, was noted by many people and was a theme of the evaluation.

Question 1:

The focus of the morning workshop was clinical trials, and looking at them from different perspectives. What are the most important things that you have learnt from the morning session?

The themes of what participants felt they had learnt from the morning session were:

- The balanced input from lay and professional participants
- The interactive nature of the morning and 'active listening' by the group
- The increased understanding of: **what** Randomised Controlled Trials (RCT's) are, **why** they are needed health research, **how** they are constructed and the challenges in planning an RCT.

Understanding the ...*"the pathway to developing a trial"* *"how to make a trial robust"*

- The different family experiences of CJD gave different perspectives on how a CJD trial might actually run in practice.

"everybody who has cared for relatives with CJD has a slightly different concerns/questions - possibly related to type; where they live; involvement in support groups"

Question 2:

The focus of the afternoon workshop was to discuss CJD trials and the PRION trial in particular. What has been most useful about the afternoon session?

The themes of what participants felt had been most useful from the afternoon session were:

- John Collinge's presentation for clarity, interest and background to CJD trials
- The literal depiction of the morning group discussions, this was felt to validate **all** of the groups input and provided a welcome structure to a lot of information

"to see/hear concerns from so many people spelled out, as a list, that can be taken into consideration, point by point when planning the trials"

- The process also demonstrated the breadth of concerns/issues expressed by lay and professional groups

- The reality of the timescales for the trial, given the nature of some types of CJD
- The challenges that designers of RCTs face, particularly with a disease such as CJD

"everyone at a high level is really thinking about the issues"

- Pat Troop's discussion session and style was positively commented on and raised important issues. Although these weren't always related to the CJD trial they helped with the bigger picture of CJD

Question 3:

Has the workshop met your expectations?

All of the respondents felt that the workshop met their expectations; several stated that it had exceeded their expectations. Of note were the several comments about the quality of listening amongst groups.

"A rare occasion - where consumers/families really feel that they have been listened to!"

Question 4:

Do you have any comments (positive or negative) about the style and balance of the programme, effectiveness of the facilitators, speakers etc

- Speakers/facilitators 'performance' ranged from good to excellent, there were no negative comments.
- Some people felt that there was too much in the day and that the afternoon group work on issues identified in the morning were too short as a result
- For afternoon group sessions, more skilled facilitators were needed
- Generally the equality of contributions for lay and professionals, (one comment about one group being dominated by a professional) and the comprehensiveness of recording the discussions and issues raised.
- The enthusiasm of all involved in the workshop

Question 5:

Do you have any comments (positive or negative) about the venue, catering and administration of the workshop?

- Generally people thought that the venue was good, with excellent facilities and refreshments.
- Some preferred the morning 'round table' style of seating aiding communication (but a bit cramped and hot) some the afternoon layout with improved Audio Visual Facilities and more space, (but less popular seating style).

Question 6:

Finally do you have any thoughts or comments about CJD trials that haven't been discussed today?

- The role of the media
- The likely time scale of the first CJD trial
- More information about the drugs 'in the pipeline' for possible CJD therapy
- A perspective from someone with CJD who may be offered a place on the trial
- The issue of consent, and the problems it may cause within a family
- The uncertainty of the CJD 'picture' at present and the need for different trial designs
- The causes of CJD and what work is going on in this area

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