NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Patient/carer organisation submission (STA)

Nintedanib for treating idiopathic pulmonary fibrosis [ID752]

Thank you for agreeing to give us your views on this treatment that is being appraised by NICE and how it could be used in the NHS. Patients, carers and patient organisations can provide a unique perspective on conditions and their treatment that is not typically available from other sources. We are interested in hearing about:

- the experience of having the condition or caring for someone with the condition
- the experience of receiving NHS care for the condition
- the experience of having specific treatments for the condition
- the outcomes of treatment that are important to patients or carers (which might differ from those measured in clinical studies, and including health-related quality of life)
- the acceptability of different treatments and how they are given
- expectations about the risks and benefits of the treatment.

To help you give your views, we have provided a questionnaire. You do not have to answer every question — the questions are there as prompts to guide you. The length of your response should not normally exceed 10 pages.

1. About you and your organisation

Your name: [redacted]

Name of your organisation: Action for Pulmonary Fibrosis

Your position in the organisation: Chair of Trustees

Brief description of the organisation: Action for Pulmonary Fibrosis is a registered charity focusing on IPF - raising awareness of the disease, supporting patients and their family members and working with local and regional patient support groups around the country. The charity also works with health care professionals to promote earlier diagnosis and a better understanding of living with the disease as well as raising funds for research. We are not a membership organisation but work with patients and family members across the UK. Our funds are raised mainly from donations and individual fundraiser activities supplemented by small corporate grants.

(For example: who funds the organisation? How many members does the organisation have?)

We are asking for your collective view as an organisation and will be asking patient experts for their individual input separately. If you have the condition, or care for someone with the condition, you may wish to complete a patient expert questionnaire to give your individual views as well.

2. Living with the condition

What is it like to live with the condition or what do carers experience when caring for someone with the condition?

IPF is a distressing, terminal progressive illness that limits physical activity due to increasing breathlessness that affects every activity one attempts. With progression of this disease, an individual's exercise capacity reduces so that a person becomes breathless even at rest, with talking and eating. This can result in weight loss and fatigue. The amount of assistance an IPF patient needs increases as the disease progresses, this may include mobility aids such as stair lift and mobility scooters, to help with activities of daily living such as washing, dressing and eating.

As well as breathlessness, patients, may have a debilitating cough. Coughing spasms can be uncontrollable and associated with symptoms such as vomiting and syncope. Intractable cough can impact quality of life as patients find this symptom embarrassing and will avoid social situations.

In addition there is the psychological burden of knowing that there is no cure. As a consequence of this progressive lung disease, patients experience anxiety, particularly associated with breathlessness, and depression.

3. Current practice in treating the condition

Which treatment outcomes are important to patients or carers? (That is, what would patients or carers like treatment to achieve?) Which of these are most important? If possible, please explain why.

A cure would be the most important treatment outcome, otherwise an intervention that slows the rate of disease progression or alleviates the symptoms of cough and breathlessness would be of great benefit to patients and carers.

What is your organisation's experience of currently available NHS care and of specific treatments for the condition? How acceptable are these treatments and which are preferred and why?

At present the only treatment is Pirfenidone, which has considerable side effects of nausea, lethargy, loss of appetite, weight loss, liver toxicity and photosensitive skin rash. It involves taking 3 tablets three times a day. To minimise side effects patients also have to use high factor sunblock daily and to have regular monitoring blood tests. Many eligible patients are unable to take Pirfenidone because of intolerance to the considerable side effects.

As Pirfenidone is restricted to patients with FVC <80% and >50% and the rate of intolerance is high, only 35% of patients are currently able to have a treatment to slow the disease.

4. What do patients or carers consider to be the advantages of the treatment being appraised?

Benefits of a treatment might include its effect on:

- the course and/or outcome of the condition

- physical symptoms
- pain
- level of disability
- mental health
- quality of life (such as lifestyle and work)
- other people (for example, family, friends and employers)
- ease of use (for example, tablets rather than injection)
- where the treatment has to be used (for example, at home rather than in hospital)
- any other issues not listed above

Please list the benefits that patients or carers expect to gain from using the treatment being appraised.

Course of disease is slowed down. Clinical trial data show that the treatment is equally effective for patients with FVC <70% as well as those with FVC >70%.

Family and carer are impacted upon as reduces dependence on them.

Mental health will improve if slows down disease progression which gives time to adapt to limitations.

Only take 2 tablets per day morning and evening and can be used at home.

Side effects are tolerable and manageable.

We expect that this treatment will improve quality of life as it slows rate of disease progression (similar efficacy to Pirfenidone) and has fewer side effects. More importantly, it has been shown to benefit those patients with early or mild disease, which we expect would result in improved survival.

Please explain any advantages that patients or carers think this treatment has over other NHS treatments in England.

It provides an alternative therapy for IPF patients who are intolerant of or unable to use Pirfenidone. This includes individuals with renal impairment as Pirfenidone is contra-indicated in severe renal impairment (CrCl<30 mls/min)

If you know of any differences in opinion between patients or carers about the benefits of the treatment being appraised, please tell us about

them.

No

5. What do patients and/or carers consider to be the disadvantages of the treatment being appraised?

Disadvantages of a treatment might include:

- aspects of the condition that the treatment cannot help with or might make worse
- difficulties in taking or using the treatment (for example, injection rather than tablets)
- side effects (for example, type or number of problems, how often, for how long, how severe. Please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)
- where the treatment has to be used (for example, in hospital rather than at home)
- impact on others (for example, family, friends and employers)
- financial impact on the patient and/or their family (for example, the cost of travel to hospital or paying a carer)
- any other issues not listed above

Please list any concerns patients or carers have about current NHS treatments in England.

There are insufficient treatments for IPF. There are no treatments available other than clinical trials, for patients who cannot tolerate Pirfenidone.

Difficulty accessing treatment for patients with mild or severe IPF, ie those patients who fall outside of NICE recommendations for Pirfenidone (ie FVC <50% or >80%).

There is insufficient access to pulmonary rehabilitation. In addition the rehabilitation programme needs to be tailored for specific needs of IPF patients.

Please list any concerns patients or carers have about the treatment being appraised.

A few patients have expressed concerns about GI side effects. For the majority of patients this is an acceptable side effect given the benefits of the treatment.

If you know of any differences in opinion between patients or carers about the disadvantages of the treatment being appraised, please tell us about them.

None

6. Patient population

Are there any groups of patients who might benefit more from the treatment than others? If so, please describe them and explain why.

IPF patients with FVC >80% or FVC <50% who currently cannot have Pirfenidone.

IPF patients who have renal impairment as this is a contra-indication for Pifenidone (CrCl <30 mls/min

Are there any groups of patients who might benefit less from the treatment than others? If so, please describe them and explain why.

Patients with very advanced disease who are in the terminal stage of their disease may have worse quality of life related to GI side effects.

7. Research evidence on patient or carer views of the treatment

Is your organisation familiar with the published research literature for the treatment?

✓☐ **Yes** ☐ No

If you answered 'no', please skip the rest of section 7 and move on to section 8.

Please comment on whether patients' experience of using the treatment as part of their routine NHS care reflects the experiences of patients in the clinical trials.

Do you think the clinical trials have captured outcomes that are important to patients? Are you aware of any limitations in how the treatment has been assessed in clinical trials?

Yes, the main outcome evaluated is rate of progression of disease as assessed by change in FVC.

Limitations - no data on impact of the treatment on cough.

If the treatment being appraised is already available in the NHS, are there any side effects that were not apparent in the clinical trials but have emerged during routine NHS care?

This treatment is also licensed for non-small cell lung cancer, but at a higher dose. There was no difference in treatment associated side effects.

Are you aware of any relevant research on patient or carer views of the condition or existing treatments (for example, qualitative studies, surveys and polls)?

✓☐ **Yes** ☐ No

If yes, please provide references to the relevant studies.

Perceptions, experiences and needs of patients with idiopathic pulmonary fibrosis http://onlinelibrary.wiley.com/doi/10.1111/jan.12587/abstract

8. Equality

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Protected characteristics are: age; being or becoming a transsexual person; being married or in a civil partnership; being pregnant or having a child; disability; race including colour, nationality, ethnic or national origin; religion, belief or lack of religion/belief; sex; sexual orientation.

Please let us know if you think that recommendations from this appraisal could have an adverse impact on any particular groups of people, such as:

- excluding from full consideration any people protected by the equality legislation who fall within the patient population for which the treatment is/will be licensed;
- having a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the treatment;
- any adverse impact on people with a particular disability or disabilities.

Please let us know if you think that there are any potential equality issues that should be considered in this appraisal.

The following issues should be considered:

1 As we have discussed above, Pirfenidone is restricted to IPF patients with FVC 50-80%. Clinical trial data demonstrated therapies were equally effective for IPF patients with FVC <70% and FVC >70%. Based upon these data,

FDA approved Pirfenidone and Nintedanib for all patients with IPF with no FVC restrictions. In keeping with this Nintedanib named patient access scheme is available for all patients with IPF (as diagnosed by ILD specialist centre MDT in accordance with ATS/ERS/BTS guidance)

2 There needs to be clear definition of treatment failure/lack of therapeutic response.

Are there groups of patients who would have difficulties using the treatment or currently available treatments? Please tell us what evidence you think would help the Committee to identify and consider such impacts.

9. Other issues

Do you consider the treatment to be innovative?

✓☐ Yes ☐ No

If yes, please explain what makes it significantly different from other treatments for the condition.

Any second treatment shown to have an effect for a condition where there are limited treatments will make a considerable impact to patients who cannot tolerate Pirfenidone.

Are there any other issues that you would like the Appraisal Committee to consider?

10. Key messages

In no more than 5 bullet points, please summarise the key messages of your submission.

- Currently Pirfenidone is the only treatment for IPF, for which only 35% of patients are eligible or can tolerate the drug
- Nintedanib provides an alternative to Pirfenidone with fewer side effects
- Nintedanib should be available to all patients who could benefit from it
- Slows the rate of disease progression and improves quality of life for patients and their carers