Health Technology Appraisal

It is obvious that without these technologies, there would be an even higher mortality rate for people who have pulmonary arterial hypertension associated with scleroderma. These medications, although not a cure, are enabling people to live longer and have a better quality of life. It is the only real hope which has been available for this serious but rare condition and is imperative that the technologies are made available to those who are unfortunate enough to develop pulmonary hypertension as a serious complication of scleroderma.

The following are comments from 7 patients (labelled A – G), regarding the drugs for the treatment of pulmonary arterial hypertension in scleroderma.

A) What is it like to have the condition?

What symptoms and problems do patients have as a result of the condition?

A) Extreme tiredness and breathlessness – not able to go out and do things I used to do.

B) Cannot do housework, visit friends or go on holiday.

C) Everything I do is an effort and I have to rely on other people to do things which has taken away my independence.

D) My life has changed from being independent to dependent on others due to the breathlessness, coping with regular medication and feeling extremely tired with no energy.

E) I can no longer do what I used to do due to breathlessness and tiredness on any exertion.

F) My symptoms are breathlessness and total lack of energy. Loss of independence.

G) Reduced quality of life – very restricted in what I can do.

How does the condition affect your day to day life?

A) Very tired and limited mobility. Very breathless.

B) Because of pulmonary hypertension I find I get breathless very quickly i.e. a simple job like vacuuming leaves me very breathless.

C) All aspects of every day living and activities e.g. household and social activities have to be carefully planned and spaced. Costs incurred by employing cleaners and gardeners and adaptations at home have to be budgeted for on a small pension and state benefits.

D) Makes me feel tired, exhausted and breathless.

E) Day to day activities are limited.

F) Considerably

G) Restricted severely.

Are there activities that you are not able to do because of the condition?

A) Walk any distance. Majority of household chores.

B) I used to enjoy ballroom dancing but cannot participate now which is very frustrating. I also find I need the aid of the trolley when shopping as I need to hold onto something.

C) Walking any distance, housework, gardening and travelling abroad. I had to retire early on health grounds.

D) Use stairs, gardening, go for walks, shop for clothes, go to exhibitions and do housework.

E) Yes

F) Yes - walking and cycling.

G) Yes

Does the condition have an impact on your family, friends and employers?

A) Much curtailed social life. Worry and responsibility for immediate family.

B) Yes. My husband has to help me around the house or whenever out ‘walking’ or when climbing stairs.

C) Yes. My mother worries a lot and at 87 helps me out! I cannot stay with or visit friends at the drop of a hat as oxygen has to be organised in advance and then I might not feel well enough at the pre-arranged time.

D) Family have to do more for me, take time off work, friends sometimes do not include me assuming that I will be unable to keep up.

E) It effects my family and I am unable to work.

F) I live on my own so this does not really apply. My friends are very supportive. I am retired.

G) Yes.

B) What are the outcomes that matter most to patients?

How does the technology compare with other available treatments?

A) Not taken any alternative medication, as yet – awaiting funding for Bosentan.

B) At present I am taking Bosentan (Tracleer) and I find it has greatly improved my health, more so than the medication I was on previously (viagra).

C) From reading the "PHA" booklet, other treatments including hospital stays, intravenous lines, nebulisers every few hours, Hickman lines, self-inserting needles seems horrendous, soul-destroying, painful, risky, time consuming and worrying. Taking one tablet a day is easy.

D) Only taken Bosentan and Sitaxentan.

E) Much better - more of an improvement.

F) The technology is fantastic - I have never tired of the treatments.

G) Helped considerably.

What positive and negative impacts does the technology have on your condition?

A) Only very positive.

B) Medication is helping my breathing and mobility. I also suffer from Raynaud's and scleroderma and although I still feel the cold - I feel my medication has helped my circulation.

C) Negative - Constant vigilance in pill taking with all other medication and being well organised in this respect.
Positive - Hope - feeling of well-being - less breathless and more energy - possibly as early days yet.

D) Reduces breathlessness.
Bosentan compromised my liver.

E) Notice much more of an improvement.

F) Only positive helping my breathing.

G) Walking better.

Which symptoms is the technology best or worst at treating?

A) Breathing and mobility much improved.
Dizziness and nausea only slightly better.

B) My medication is definitely helping my breathing. It has caused fluid retention and swelling of the legs and ankles for which I am taking water-tablets.

C) Best - feeling of well-being and breathlessness.

D) Best - breathlessness.
Atrial fibrillation or palpitations aren't impacted by drug.

E) Best at treating breathing at the same time as improving mobility.

F) Bosentan has helped a lot with my breathing.

G) Best for breathlessness. Tiredness improving.

What difference does the technology make to your long term health and well-being?

A) Without treatment I would be totally incapacitated as I was at the time of diagnosis.

B) It has improved my lifestyle somewhat.

C) Hope - Greater life expectancy. Sitaxsentan has made a big difference to my well being so far (only been on 4 weeks).

D) Don't know.

E) It has made a considerable difference in improving my health. In turn making me feel better and able to cope with day to day living.

F) All the difference in the world. My daily oxygen is a lifeline.

G) Getting improved mobility.

Does the technology have an impact on others - e.g. family, friends, employers?

A) A degree of returned independence relieves the responsibility and worry to my family.

B) No answer

C) Medication doesn't have an impact on others, except that they're pleased I'm feeling better than I was.

D) No.

E) No

F) None

G) No

Does the technology have any side effects? If so which ones are you prepared to put up with and which ones do you find unacceptable?

A) No side effects that I cannot cope with.

B) The medication has caused fluid retention and swelling in legs and ankles for which I am taking water tablets.

C) I'm not sure yet. Bosentan gave me headaches. I could put up with these but it was nice when they stopped! Sitaxsentan hasn't given me headaches and the first blood tests have not yet been done.

D) Sitaxentan - none so far.

E) No side effects.

F) No

G) No

What would be the impact on your condition if the technology was not available?

A) I would not cope for very long.

B) It would be unthinkable. I certainly would not be able to walk very far and would probably become housebound.

C) Hopeless, dire consequences and what would happen to my mum if I wasn't here?

D) Very breathless - less able to look after myself i.e. cook, shop (for food), bathe and dress and visit friends.

E) My condition would most definitely deteriorate.

F) A great impact - my life would be more than difficult.

G) Catastrophic.

C) Using the technology

How well or badly does use of the technology fit into your life? e.g. do you have to go to the hospital to receive the medication or is the medication administered by someone else?

A) Medication prescribed by consultant with 6 to 8 weeks check.

B) Fortunately my medication is sent by courier and is in tablet form.

C) The pills are given to me by the hospital. The Bosentan was delivered by courier. I can take the tablets easily. I have to travel to London (at the moment) for the tablets.

D) Medication sent by post or issued on clinic appointments.
Have to go to GP surgery monthly for blood test monitoring.

E) Medication fits in quite well and is administered by the hospital.

F) Not applicable

G) Medication self administered.

Is there anything about the technology that makes it hard or easy to use?

A) Medication is not a problem.
Oxygen concentrator 15 hours per day has taken a little getting used to.

B) Unfortunately I have to have a blood sample every month sent to the hospital to make sure my liver count is ok.

C) The pills are easy to take in a blister pack, once a day.

D) Blister pack of Sitaxentan hard to access.

E) No

G) No

Do you have any difficulty using the technology?

A) Oxygen concentrator can be inconvenient and restrictive sometimes.

B) My medication is in tablet form and I have no problem taking this.

C) No

D) Hard to access tablets from blister pack.

E) No

F) No

G) No

Is any cost involved to your family in using the technology including time, transport costs and carer costs?

A) No answer

B) Yes. I have to travel to my hospital in Sheffield every 4 months for further tests. This is time consuming and also expensive as we are senior citizens and I certainly could not make this journey without my husband.

C) Train and taxi fares to London.

NB. I have referred only to PHT medicines i.e. Bosentan and Sitoxentan not other medication including diuretics, oxygen, heart pills etc.

D) No

E) No

F) No

G) No