Scleroderma Healthcare Report

'Once you start moving, the road comes into existence'

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Sclerodermie Advies & Voorlichting

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Executive Summary

- Raynaud's phenomenon, joint complaints and esophageal problems are the most prevalent complaints/symptoms prior to diagnosis.
- All patients (except 1) started at their GP. One third of the patients were referred correctly to a rheumatologist.
- Standard tests include: blood and urine analysis, lung and heart function test.
- Mean time to diagnosis is 3.3 years.
- The most prevalent symptoms after diagnosis are Raynaud's phenomenon, fatigue and skin induration.
- Patients suffer most from fatigue, Raynaud's phenomenon and Digital Ulcers.
- The most common treatments include: Iloprost and Endoxan infusions and MTX.
- More than half of the patients see their rheumatologist every 3 months.
- Patients search for and find information at their treating physician and by contact with other patients.
- Most patients have indicated that their greatest needs are contact with other patients and effective medication for Digital Ulcers.
- Digital Ulcers are turning out to be life-controlling.
- Some patients do not receive medication for Digital Ulcers.

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Content

Introduction

Sclerodermie Advies & Voorlichting (Scleroderma Recommendation & Information - SAV) initiated the Scleroderma Healthcare study in 2013. The principal reason was that the owner of SAV, Ramona Kanters, suffers from this disease and has made it her life objective to improve healthcare and quality of life in scleroderma. In this setting we interviewed up to 200 patients and administered a questionnaire. The questionnaires were analyzed for 65 of these patients, 59 female and 6 male.

In 2014, SAV expanded the questionnaire and added additional questions. To achieve this, patients were contacted again and patient meetings were organized. To date, 21 have been contacted, 19 female, 2 male. Experience has shown that patients with more disease symptoms are quicker to seek help and support from patient forums and meetings. We therefore feel these patients are highly representative for our target group. Seeing specific questions on Digital Ulcers (DU) were included and not all patients have this disease, we split the patient group.

The study was subdivided in:

Part 1: analysis of 65 patients with answers to the following questions:

1. Which complaints/symptoms did patients have prior to diagnosis?
2. Which doctors did patients see prior to diagnosis?
3. Which tests were performed in patients?
4. How long did diagnosis take (until SSC was confirmed)

Part 2a: analysis of 21 patients with answers to the following questions:

5. Which symptoms (or complications) do SSC patients have?
6. Which symptoms (or complications) are most bothersome to patients?
7. Which treatments did patients receive?
8. What is the follow-up strategy (how often do patients see their doctor and for what)?
9. Where do patients search for and find information?
10. What do patients need most?

Part 2b: analysis of 13 patients with answers to the DU-specific questions:

11. How do patients experience DU versus other symptoms or complications?
12. How do patients measure the effects of medication (particularly for DU)?

The attached Excel file contains the questionnaire outcomes.

Part 1: analysis of 65 patients

1. Which complaints/symptoms did patients have prior to diagnosis?

The most prevalent complaints/symptoms before diagnosis include:

1. Raynaud's phenomenon: 63.1%
2. Joint complaints other (rheumatic complaints): 46.2%
3. Esophageal problems: 41.5%
4. Swollen hands: 40%
5. DU: 38.5%

Other complaints (in order of prevalence) include: skin induration, intense fatigue, general malaise, stiffness in the joints, tingling sensation in fingers ('numbness'), muscular pain, facial change, tightness in the chest, joint pain in the wrists, weight loss, itch, diarrhea, smaller mouth opening, pigment discoloration, swollen/distended body parts other, poor dental health, extreme birth marks, frequent and dry coughing, stroke, local numb feeling, deafness, body deformation, eye impairment, difficulty swallowing, leukemia.

A number of these complaints likely have nothing to do with scleroderma.

Many patients have the same complaints/symptoms! We may conclude that earlier diagnosis is possible, arresting the disease in an earlier stage, provided the GP is better informed …

2. Which doctors did patients see prior to diagnosis?

Everyone started at their GP, except 1 person who was already being treated by a hematologist for leukemia.

Almost 31% were referred directly to a rheumatologist, who then made the correct diagnosis.

23% of the patients were referred to a rheumatologist directly after their visit to the 1st specialist, after which the correct diagnosis was made.

The specialists correctly referring patients with or without suspicion to a rheumatologist are: internist (5x), neurologist (4x), pulmonologist (2x), GP, endocrinologist, liver specialist and vascular surgeon.

Well over 30% returned to their GP after their visit to the 1st specialist. This occurred for several specialists; remarkably, some rheumatologists were unable to make the correct diagnosis, almost 5%.

29% of the patients were referred by the 2nd specialist to a rheumatologist (3rd specialist), after which the correct diagnosis was made. That GP now refers to the correct specialist (22%).

11% of the patients needed a 4th specialist to get the correct diagnosis, 3% a 5th specialist and 2% a 6th specialist.

After diagnosis, all patients were treated by a rheumatologist. Additionally, 14% were treated by a pulmonologist, oncologist, neurologist, cardiologist, hematologist, ophthalmologist, dermatologist, and/or psychiatrist.

Providing GPs with good education and correct tools (capillary microscope) would maximize benefits! Seeing most patients are referred by their GP to a neurologist (26%) or internist (23%) (in addition to the rheumatologist), this is the next best option for maximum benefits.

3. Which tests were performed in patients?

All subjects had standard blood/urine tests. Additionally, most patients had a lung function test/scan (93.8%) and heart function test (90.8%). About half of the patients had esophageal tests/scans (58.5%) and exercise testing (50.8%).

Other tests included (in order of prevalence): various scans (including isotopes), skin score measurement (Rodnan skin score), skin biopsy, nailfold capillary microscopy (30.8%), swallowing test, renal function test, muscular biopsy, alternating hot and cold baths, intestinal tests, gastric tests, muscle measurement, ECG, bone density measurement and fecal analysis.

After diagnosis, extensive tests were performed in all cases. Patients found this very encouraging. It gives them the feeling they are taken seriously, and concerns about organ involvement may be removed. Patients get a clear picture what form they have.

4. How long did diagnosis take (until SSC was confirmed)

The shortest time to diagnosis was 2 weeks, the longest 26 years. These are the exceptions; mean time to diagnose is 3.3 years.

Part 2a: analysis of 21 patients

5. Which symptoms (or complications) do SSC patients have?

The most prevalent symptoms or complications patients experience include:

1. Raynaud's phenomenon (85.7%)
2. Fatigue (76.2%)
   Skin induration (76.2%)
3. Smaller mouth opening (66.7%)
4. Digital Ulcers (61.9%)
5. Esophageal problems (gastric problems) (38.1%)

Other symptoms or complications (in order of prevalence) include: stiff painful joints, dry tight skin, loss of muscle strength, itch, difficulty swallowing, pain in lower legs, shortness of breath, ankylosing joints, limited motion, general malaise, pain in hands, Sjögren's syndrome, back complaints, impaired walking, intestinal problems, feeling of vacuum, DU in legs and feet, tightness in the chest, joint inflammations, heart and lungs affected.

Here again we see very clear symptoms emerging. The symptoms before and after diagnosis correspond reasonably. Remarkably,

- **there are more Raynaud cases after diagnosis, perhaps it was not recognized as such before diagnosis?**
- **Extreme fatigue increases after diagnosis. As the disease progresses, fatigue increases.**
- **Skin induration worsens as the disease progresses.**
- **Smaller mouth opening worsens after diagnosis.**
- **The number of DU cases increases after diagnosis. Esophageal problems decrease after diagnosis. In many cases, gastric acid inhibitors are used for symptomatic control immediately after diagnosis.**
- **After diagnosis the number of Raynaud cases increases also, but also the number of DU cases: cause-effect due to poor perfusion?**

6. Which symptoms (or complications) are most bothersome to patients?

Patients indicate they suffer most from fatigue (57.1%), secondly Raynaud's phenomenon (38.1%), and thirdly DU (33.3%). The other symptoms or complications have significant lower impact on quality of life.

Note: Of DU sufferers, 53.8% indicate this is what bothers them most.

Fatigue causes problems for very many patients. Suddenly your battery is low! The outside world is not very understanding, particularly if the devastating body changes are not visible yet.

Also the energy that Raynaud costs, is often underestimated! Patients must always remember to bring protective clothing to ward off a Raynaud episode. Additionally, stress plays a major role in Raynaud activity, and stress is part of many people's daily lives. Stress increases also if you know that this Raynaud episode is further diminishing your prospects of a cure from active ulcers.

That DU is placed third in this list, while not every one of these 21 patients have DU, is a clear sign of the impact of this problem. This is addressed in more detail in part 2b question 11 on page 7.

7. Which treatments did patients receive?

A total of 17 patients received 34 treatments, 4 patients received no treatment.

The most common treatment is Iloprost infusions (23.8%), followed by Endoxan infusions 6x and MTX (19%), Endoxan infusions 12x, stem cell transplants and Cellcept (14.3%). Depo-medrol injections (prednisolone) and plasmapheresis follow with 9.5%; some individuals received nifedipine and various alternative medicine (homeopathy, bioresonance and orthomolecular) as treatment.

Seeing Iloprost infusions came in 1st place, it would seem that treating physicians wish to ensure good perfusion to minimize the impact of Raynaud episodes, thereby preventing active ulcers or improving their healing. The administration of Endoxan and MTX attenuates the activity of SSC.

8. What is the follow-up strategy (how often do patients see their doctor and for what)?

All patients are monitored by a rheumatologist except one, she is seen quarterly by an internist. She is also the only one who is monitored by an internist. Those monitored by their rheumatologist always have a skin score test. Well over half of the patients (52.4%) are monitored every 3 months, other patients every 4 to 6 months (33.3%); some individuals say they go every 6 weeks. One patient indicates she sees her rheumatologist weekly for active DU.

In addition to rheumatologists, patients see the following specialists:

1. Cardiologist, function test once annually (85.7%)
2. Pulmonologist, function test once annually (74.4%)
3. GP, depending on nature of complaint (66.7%)

Multidisciplinary healthcare information events ('zorgpaddagen') are organized once annually, e.g. in Leiden University Medical Center LUMC; 19% attend these events. 14.3% consult a rheumatology nurse (3 or 6 have monthly follow-up), 9.5% have lung function tests every 3 to 6 months, and 4.8% have quarterly dermatology monitoring.

After diagnosis, support and follow-up may be termed adequate. If SSC becomes active, regular testing ensures quick intervention.

9. Where do patients search for and find information?

All patients ask for information from their treating physician and during patient meetings. 95.2% also search for information using a search function on the Internet and social media, such as patient forums. A small number (19%) attend multidisciplinary healthcare information events in the LUMC and 9.5% attended the world scleroderma conference.

Patients indicate they get most information from their treating physician, 74.4%. Second are the multidisciplinary healthcare information events in LUMC with 14.3%; 9.5% indicate they found most information using Internet searches.

Most patients devour any information wherever they find it. Unfortunately, this information often has a negative tone. People do not place information on e.g. patient forums until something is wrong with them. Internet searches often lead people to negative rather than the positive vibes which would be so welcome to them.

Fortunately, most patients indicate they get most of their information from their treating physician. This is not 100%; patient interviews demonstrate that physicians are still revered and are not considered very accessible. Also, patients experience pressure (rush) during consultations, causing them to forget questions (the waiting room is full). They often do not realize this until they are outside again and they have to wait until their next visit for another opportunity. Getting information by Internet searches or contact with other patients feels a lot more comfortable.

10. What do patients need most?

The majority of patients indicate that what they need most is contact with other patients, 28.6%. Remarkably, this is followed (with 23.8%) by effective DU medication, even though not all these people have DU. This is followed by understanding, recognition, explanation and clarity, more information (nutrition, tools, etc.) and symptomatic control with 14.3%. Awareness, increase energy level, effective Raynaud medication and honesty of doctor follow with 9.5%; some individuals need pain control, SSC medication and positivity.

Contact with other patients was shown to be the most important for patients. This is not surprising. The Scleroderma Netherlands Foundation has been organizing patient contact events since March 2014. Attendance is increasing. In view of the high attendance, events will also be organized in Amsterdam and Utrecht. Patients find fellow sufferers, understanding, recognition, explanation by invited experts etc. These are golden hours with smiles and tears.

It would be wonderful to make a site like 'The Face of Scleroderma' interactive.

Notably again, effective DU medicine takes second place, whereas not even all patients have them.

Part 2b: analysis of 13 patients, specified by DU

11. How do patients experience DU versus other symptoms or complications?

All patients term DU as very painful, virtually all (76.9%) indicate wound healing is poor which causes them much problems. Notably, 23.1% indicate active ulcers control their lives.

Furthermore, people indicate they have trouble during cold periods (15.4%), that the other symptoms are dwarfed by DU (15.4%), that healed spots remain very painful (7.7%), and that it is extremely tiring (7.7%).

As indicated previously, 53.8% of these patients say DU is the worst symptom of everything associated with scleroderma. The problems caused by DU in patients are truly enormous, but patient interviews show that these complaints are underestimated by treating physicians. Patients lose heart because of the pain and the hopelessness of their situation, also because of the poor wound healing.

Literal quotes from patients with active ulcers:

"My finger amputation was not as painful and tiring by far as an ulcer"

"DUs are severely annoying. They limit my daily life, the pain is very severe and I am very frustrated"

"It occupies me all day... Is it getting any better? Will it not become infected? It goes through my head all the time ... the lack of understanding is huge also. People don't understand that if you bump your finger, this does not feel as simply bumping your finger! It feels like your nerves are exposed. It makes me very tired and sad, because it all seems so hopeless!"

"The pain cuts me to the bone all day"

"I asked my treating physician to amputate the ulcerated phalanx, surely that must be less painful"

"When I bumped my finger to the edge of the table, the pain made me faint"

Doctor: "I am very sorry for you, but to us this is of secondary importance"

12. How do patients measure the effects of medication (particularly for DU)?

To give a full answer, patients were also asked which medication was/is used. 5 patients indicated they do not use medication for DU.

The 8 remaining patients use the following medication:

1. Iloprost and Bosenta (each 38.5%)
2. Nifedipine (23.1%)
3. Bosentan and Ketensin (each 15.4%)
4. Sildenafil and Ginkgo Biloba (7.7%)

Unfortunately, half of the patients indicate medication has poor efficacy. 25% of the people indicate their DU is controlled with Bosentan, 12.5% controlled with Iloprost, and 12.5% have no response.

Two patients report side effects, both including headache.

That 5 people do not receive medication for their active ulcers is very regrettable. Again, the treating physician does not take DU seriously. It does not impact life expectancy. They forget it most certainly impacts quality of life!

The good news is that 40% of the patients who actually use Bosentan, say it controls their DU. 20% of Iloprost users say it controls their symptoms.

Conclusion:

Based on our study results, there is much room for improvement in scleroderma care. Especially knowledge among GPs is suboptimal, causing diagnosis to take a median 3.3 years; the disease may be latent for long periods of time and cause irreversible damage! Once the right rheumatologist has been found, disease monitoring is adequate.

It was also shown that the problems patients experience from DU are greater than believed at first. Treating physicians do not give these problems sufficient priority as they do not impact life expectancy. There are even patients who receive no treatment whatsoever for their DU!

The relationship with treating physicians is often good, but reservedness obviously prevails. Patients do not dare ask everything or do not get to it because there is not enough time for them.

In addition to their treating physician, patients search for and find much information by Internet searches and they get much support and recognition from other patients.

By our very personalized approach and individual attention, we have won the trust of many patients. Among these are no less than 50 DU patients, who indicated they will be happy to collaborate in a follow-up study.