Rare Kidney Diseases



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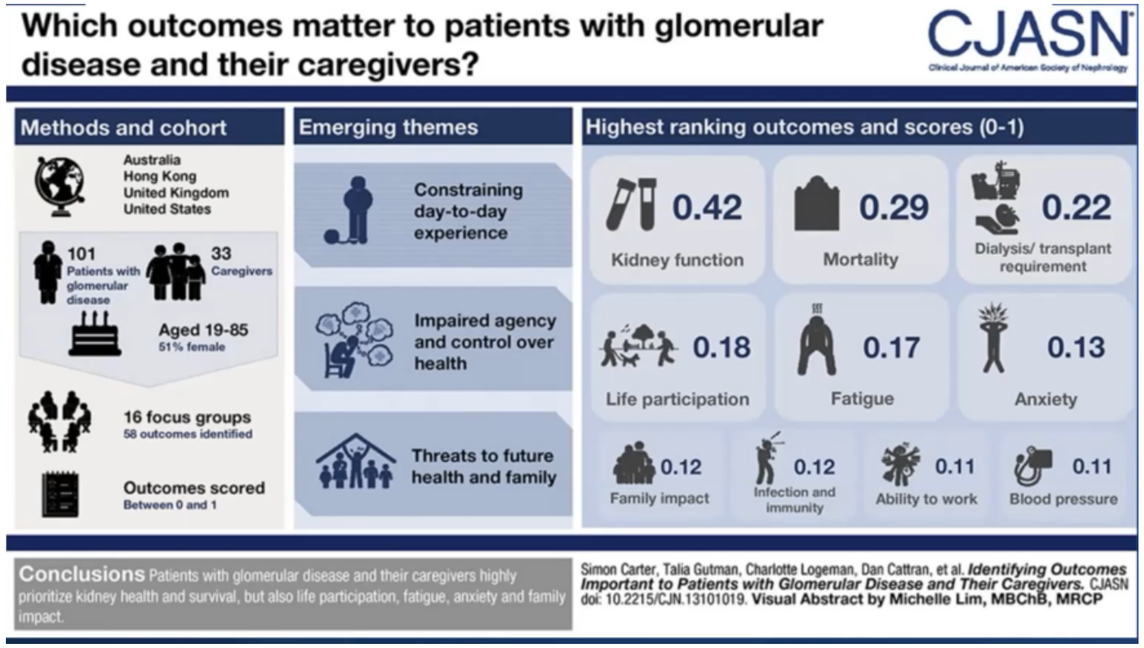
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IgA nephropathy it is the most common form of primary glomerulonephritis globally.

1. 10% of patients on dialysis have IgAn
2. up to 50% of patients with IgAN develop ESKD and require dialysis.
3. There are 200,000 people with IgAN in the European Union
4. Rare disease all together form a big disease burden across the world.

Leicester IgA Nephropathy Group is a very vibrant patient group with:

- YouTube channel in which we post range of resources for patients and different videos with information such as how to manage symptoms.

- Facebook group and it allows patients to interact. This is a great way to be able to disseminate information and for patients to be able to contact others.

Dr. Jonathan Barratt

"There were patients with rare diseases that had never met another person with the same disease"



Dr. Jonathan Barratt
Leader of the Renal Research Group within the College of Life Sciences, University of Leicester

We have been very interested in trying to understand what is important to patients, and for that we used some research methods to identify what patients are talking about on social media.

There were patients with rare diseases that had never met another person with the same disease, and being able to find somebody with the same condition allows them to share their experiences.

Symptoms seem to be a big concerned to them. Which is interesting, as this is an asymptomatic disease, so this shows that clearly there is a complete disconnect here.

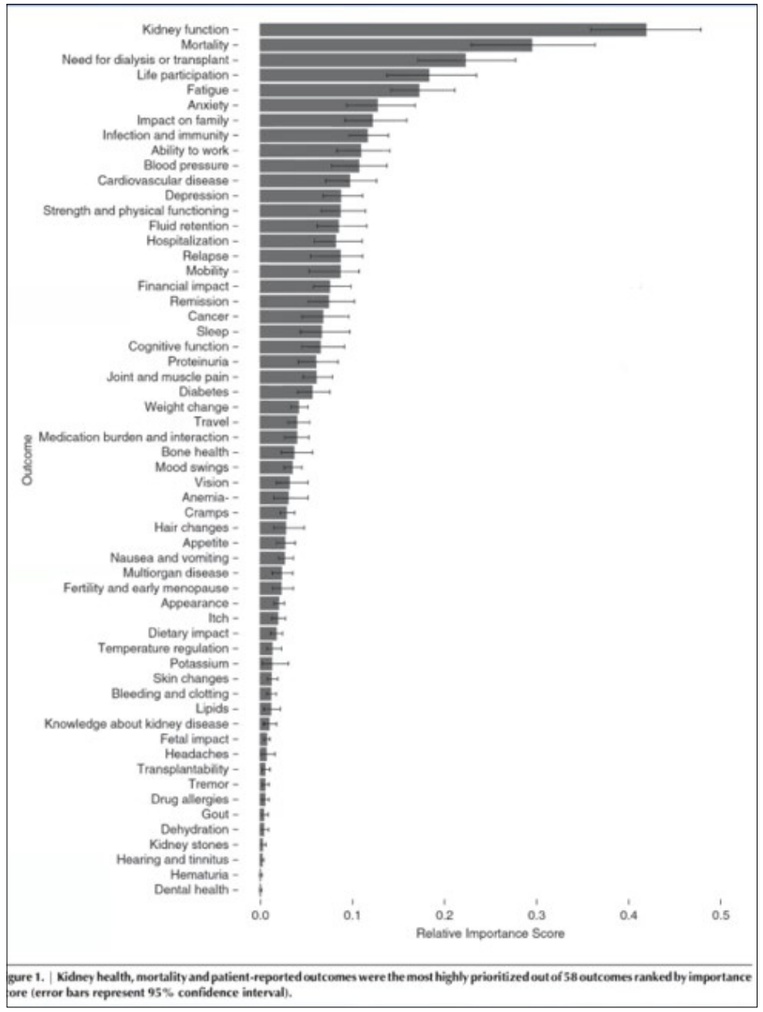
We had dieticians, psychologists, doctors, etc., answering the questions that came up. This is a working progress and one of the ways we can cover some of the unmet needs.

The National Kidney Foundation, from the US, also carried out a survey to hear the patients, which was very similar to the outcomes reported in the UK: being tired, the “brain fog”, the anxiety, and how all this impacts the life and the social activities.

Another interesting initiative is SONG initiative, which is about asking patients what is important to them when testing a new drug. This is showing us what the priorities are for patients. We want to be able to incorporate this into how we design studies to see if a treatment works. Patients want to know that kidney function is getting better and they are going to live longer. But actually, life participation, fatigue and anxiety are things we wouldn’t normally think about when we are assessing a new treatment for disease. This are things that are significantly important for patients with rare diseases.

It is also important to empower patients and to make sure they understand what type of help is available and how they can find it. It is absolutely necessary for patients to find the doctors specialised in rare diseases.

It is true that we can not train every doctor on every rare disease, but we have to make sure the patients can find the right doctor.



gure 1. | Kidney health, mortality and patient-reported outcomes were the most highly prioritized out of 58 outcomes ranked by importance ore (error bars represent 95% confidence interval).

The best people to tell us the humanistic burden of rare kidney diseases are the patients. As doctors, we must try to better understand the burden of the disease to improve the care we give to patients.

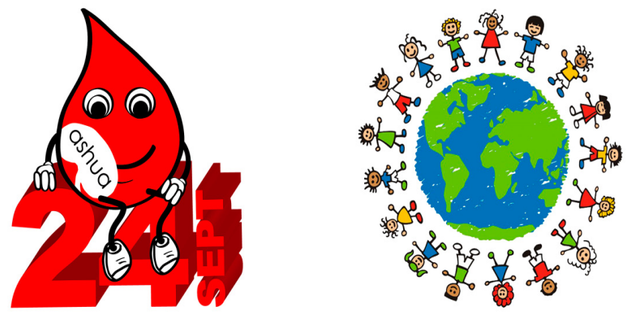
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Francisco Monfort

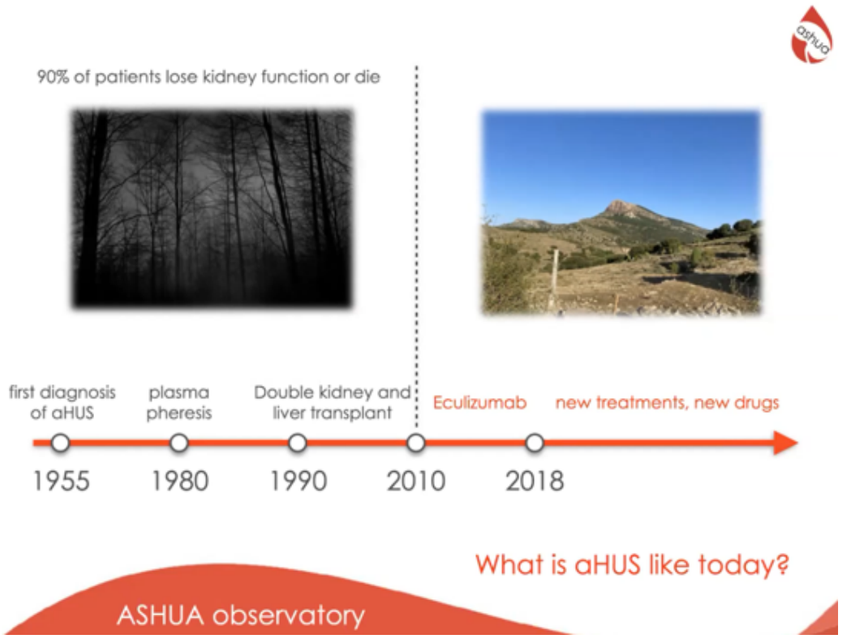
“From the association we try to encourage more human relations”

ASHUA was born in 2012 as a support platform to inform and advise patients with this disease and their families. We collaborate with nephrology societies in addition to other patient associations such as ALCER, FEDERG and ERKNet.

ASHUA Observatory is a tool that shows us that research and training of doctors saves lives. It is the largest study conducted by a patient association on this rare disease.



24 Septiembre #shua24sept
Día internacional del SHUa

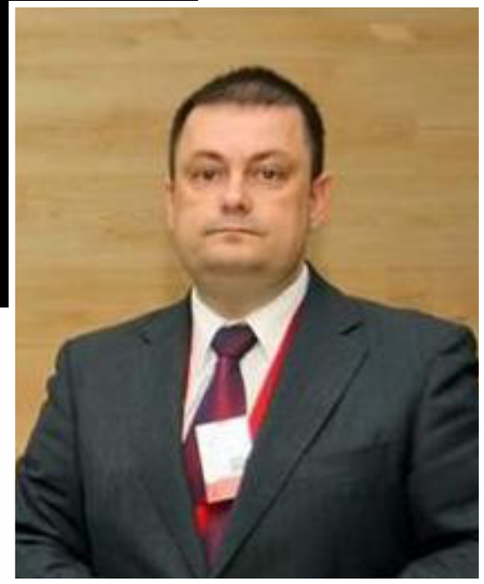


Some data about aHUS:

1. 66% of aHUS patients are woman, and this is due to special triggers for women such as pregnancy, oral contraceptives or childbirth.
2. Less than 1% of the family members could debut on the disease.
3. 22,4% of the patients were diagnosed in less than 48 hours, and 26,5% within the first week.
4. Before 2010, it took around 3 months to be diagnosed. Nowadays patients in general only consulted 2 doctors before they had the final diagnosis and this shows that doctors are well trained.

This is an image that shows how the aHUS has evolved. The good management of this disease by the doctor and an effective treatment reduce the number of episodes. Patients usually suffer 2 episodes of aHUS in their lifetime. It is very important to learn about the genetic variants of each patient.

aHUS observatory results:



Francisco Monfort
President of ASHUA (Atypical Haemolytic Uremic Syndrome Association)

- 47,4% chronic kidney disease, 42,1% dialysis and 30,1% none, which is very good.
- Impact on the lifestile, 44,4% of patients answered that it hadn't affected their life. 17,5% answered job loss, and 21% education loss, so there are still things to improve.
- Patients were very (29%) or quite (45%) satisfied with the information they had received from doctors, so this shows how doctors are trying to offer patients all the information they seek
- Also, Patient Associations were a very important source of information to patients (72%)

We are working closely with researchers, because it is very important for them to understand the real problems of patients. It is also important to raise awareness, for example during the awareness days. And also, we need to build good relationships between doctors and patients. From the association we try to encourage more human relations.

Tess Harris
Moderator

"It is a very exciting time for rare disease patients"



Tess Harris
Moderator
President of FEDERG (European Federation of Patient Groups affected by Rare/Genetic Kidney Diseases)

It is a very exciting time for rare disease patients. A month ago, the UN adopted a resolution on addressing the challenges of people living with rare diseases and their families. This recognizes that over 3 hundred million people are living with rare disease and a large number of those will have kidney disease. It is also recognising that rare diseases affect the families and the loved ones of the patients. It is a call to action of all UN countries to focus efforts and investments on rare diseases.

Another important event is the first rare and genetic diseases drug development conference which has brought together pharmaceutical industries and regulators to discuss how the latest scientific research has been applied and the development of new therapeutic treatments in many conditions.