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# Patient Voice

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## Patient Voice

The term Patient Voice is often used in the context of patients and health care professionals learning from the trials and tribulations of navigating through the process of illness in search of quality care, answers and healing- from patients who have been there and done that. Teach patients to raise their voice about their own care and treatment plan.

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United Parent Projects  
Muscular Dystrophy

## Patient Voice

In the view of the NHS patient's voice means: Putting the patient at the centre of the NHS. On their website you'll find: 'The NHS Plan contained a raft of reforms designed to do this. Eventually, say ministers, this could lead to patients having far more say in the way that the health service is run.'

## Patient Voice

The Dutch institution for Care and Medical research (ZONMW) states:

People with a disease or a disability have a weaker position than other parties in care such as professionals, insurance companies, pharmaceuticals and researchers. That is remarkable. Care revolves around the people with a disease or disability. Without them there would be no need for research and care policy.....



## Patient Voice

.....It is important that the needs of these groups are the starting point for initiatives concerning them. There is a lot to win if their experience and expertise is utilized. People with disabilities and diseases know what it means to have this condition. It means they will bring in a different perspective to caregivers, researchers or policymakers. Their questions and needs are based on their own experiences, interests and vision.

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## Patient Voice

Is about specific experience and expertise which should be included in individual care, drug development, laws and health care systems.

In a clinical trial network this includes:

Input from patients in clinical trials from the start

Input from patients in standards of care and individualized care

Patients as steering partners in health care systems

Patients being advocates for patients rights and ownership of data.



## Patient Voice and Clinicians

Different parties in relation to care, research, clinical trials and drug development have different expertise. Clinicians, researchers, industry and patients all have a role.

Clinicians have often seen a lot of patients and could and should be advocates for patients. In fact that is included in their oath when they become a medical doctor.

## Patient Voice and Clinicians

So the clinicians' voice should be based on what is best for the patients in their opinion, however this should not be confused with the patient's voice as the patients may have a different opinion based on their position and their experience.

Their priorities in relation to care, clinical trials and other parts of research may differ from the clinicians ideas.



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# Patient Voice in Clinical Trials

Outcome measures

Burden

Risk

Benefit

Trial design

Information

Inclusion Criteria

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# Patient Voice in Research

Setting priorities

Research agenda

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# Patients Voice - Standards of care

Bring in their expertise

Identifying gaps

Setting priorities

Provide information

Lay language, tune and timing

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# Patients voice - Rights

Access Care

Drugs (Compassionate use / Reimbursement)

Ownership of data

Registries

Biobanks

Clinical trials

Information

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# Children

The moral agency of seriously ill children should not be underestimated.

In order to be good advocates for themselves, children should be well informed and given the opportunity to speak!

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## Patient organisations

Historical the role of patient organizations was to bring peer sufferers together.

The position from patient(organisation)s has changed from 'sufferers' and users of care to partners in care, research and drug development

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## Voice of Patient organisations

NMD patient organisations did more than raising their voice. They came forward to organise research meetings where they made it clear they were willing to shoulder responsibility and contribute towards advancing treatments and a cure.

Some organisations started their own research institutes others invested in extramural research, clinical centers and industry to develop viable treatments for neuromuscular disorders.

## Role of Patient organisations

Patient organisations became funders and experts in the field of research and clinical trials. Their members participate in clinical trials and will ultimately be the ones to decide whether the tested drugs are beneficial to them to an extent that EM(E)A and FDA allow these drugs.



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## Role of Patient organisations in drug development

Basic research: Funders and organisers of meetings and collaborations

Translational Research / Clinical trials

Databases /Registries/Biobanks

Regulatory issues

Health insurance issues to make the drugs available before and after they are 'approved for the market'

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## Advocacy in drug development

New drugs available for as many patients as possible  
(age/mutation/country)

Outcome measures for all ages

Neonatal screening and proper DNA diagnosis

Asked regulators how to move forward with trials to avoid waisting of  
precious time

Avoid unnecessary delays and hurdles (upscaling vectors/lack of natural  
history data)

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## Lobbying (government)

More money for Care

More money for Research

Regulatory Issues

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## Lobbying (government)

Using lobbyists

Patients going to the house of parlements

Patients writing to their members of parlement

‘Activist’ activities

Being member of governmental committees

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## Role of Patient (organisations) in clinical trials

Trial design (burden/outcome measures)

Recruiting patients /registries

Members participate

Funders

Information

# Patient organisations - Information

Translate into lay language

Information/data clinical trials available for patients before publications

Convince researchers and industries to use comparable outcome measures

Management of expectations

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## Expectations

Give your input during these 2 days  
Fill out the Treat-NMD consultation form