

## Side-effects of new treatment options

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# Side-effects of New Treatment Options

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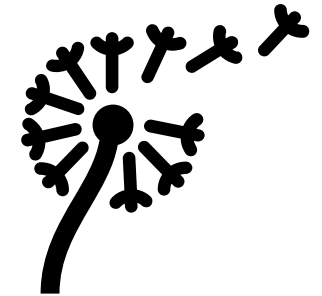
9th Endo-ERN General Assembly (GA)

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Copenhagen, DK

# Time before treatment options for ACH

- Focus on acceptance, individual medical aspects
- General acceptance seeing a specialist once a year
  - Also possible on the annual conventions of my organization
- General agreement on centralized care
  - Doctors, organization and community!
- Interest in potential pharmaceutical options was high
  - Update at every annual meeting
  - Self-medication with off-label substances



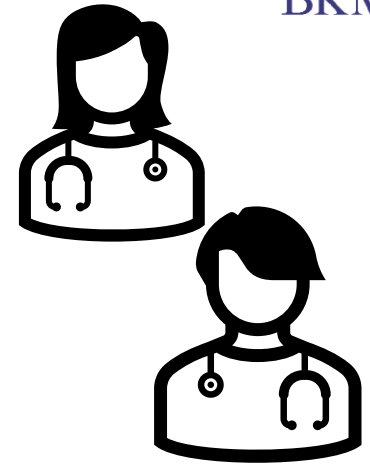
# Visible effects after 3 years

- Acceptance is very high
  - Few side effects
  - Differences in response to treatment
- New interest in the condition
  - More research
  - More treatment options are in development
- New medical burden
  - Label requires visits every 3-6 months
  - Many clinics want closer monitoring



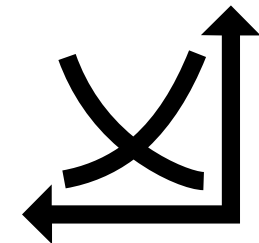
# Side-effects on clinical management

- Centralized care is being questioned
- Local clinics start treatment
- Especially in smaller clinics
  - Absence of binding protocol results in very different care
  - Unnecessary examinations are increasing
  - Some clinics reach out to us to get information
  - Focus on height in medical communication



# Side-effects on families

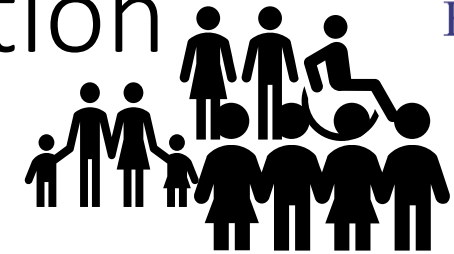
- Immediate start of treatment reduces possibility to cope
- Treatment is sometimes regarded as „cure“
  - Possibility to ignore the condition
- Response is compared in social media
  - Misinterpretation of data (absolute/relative growth)
  - New pressure on families
- Focus on height neglects other needs





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# Side-effects for my patient organization



- Generation before/after treatment option
  - Participant at our conference to his younger child: „Take the drug so that you don't have to look like them.“
- Expectations of parents are very high
  - Hope vs. available data
  - Imagination of average height parents regarding dwarfism
- New families feel less need to connect to the community
- Reaching out to more clinics and paediatricians adds workload on us

# Conclusion

- Availability of the first pharmaceutical treatment option has changed our landscape tremendously
- There are many positive issues, such as research
- Some side-effects have a potential to worsen existing care
- Additional treatment burden increases already high pressure of the families

