



University
of Basel

Department of
Clinical Research



University Hospital
Basel

The role of patient organizations

Efficiency Strategies for Clinical Studies

Therese Stutz Steiger, 06.07.2017

PRORARIS



Alliance Maladies Rares - Suisse
Allianz Seltener Krankheiten - Schweiz
Alleanza Malattie Rare - Svizzera

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1 Some personal information1

- Born with a rare disease
- Characterized as a chronic condition
- Irregular acute episodes
- Health problems in childhood
- “New” health problems by getting older

- Three decades of very few bone-related problems

- Trained as an MD with an MPH



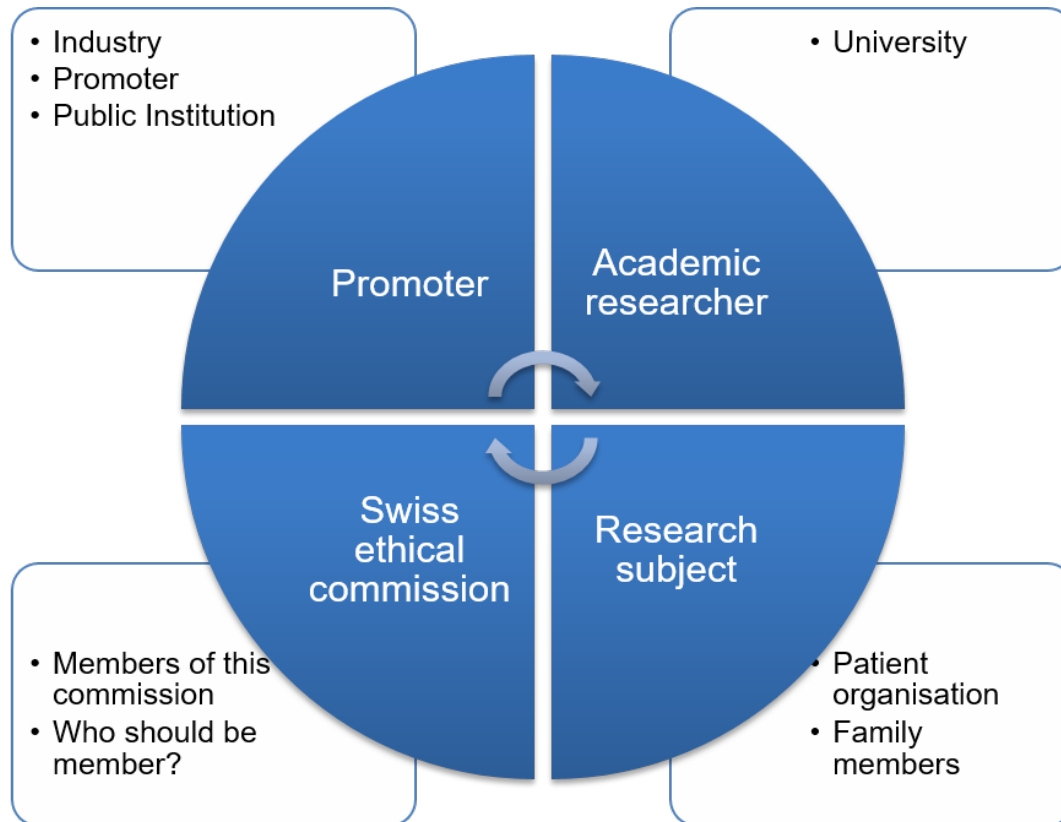
2 Patient and consumer organisations in Switzerland

- Umbrella organisations of affected people and organisations
- Disease related patient organisations and support groups
- Supporting- and counselling organisations
- Ombuds centres for patients

2 Definitions (patients perspective)

- **Clinical trial:** a research project which aims to evaluate the effects of an intervention in connection with health
 - clinical research \neq clinical care
- **Role of the person in charge of care:** individual care of the patient by respecting his needs
- **Role of researcher:** to gain knowledge which can be disseminated. The interests of the patient are not always identical

2 Partners in a clinical trial



2 Benefits

No guarantee; the study is aimed at evaluating the real topic

To consider

- Study phase
- Study methodology (placebo)
- Existing data
- Discussion with other participants

2 Risks

Some unexpected events can happen:

- in relation to the intervention
- in relation to the tests during the clinical study

To take into consideration:



A clinical study has to evaluate the safety of a therapeutic intervention. Not all risks can be eliminated.

2 SCTO Forum Clinical Research 2015: General consent–Yes but how?

“Patients often have a huge interest in clinical research as they know first hand how life changes with a specific diagnosis. They need cures and hence, the majority of them has a positive attitude towards research. However patients want to become addressed and involved.”

Karin Holm; representative of a patient organisation

3 Rare Diseases

Definition: 1 or less persons affected in 2000 persons

FOPH: 7.2% affected in Switzerland/ in total 580'000 (more than people with diabetes)

6'000-8'000 rare diseases are known

80% are genetic; in 50% of the cases the responsible gene is known

>50% in childhood

95% no specific treatment

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Verein Schweiz



SMCCV

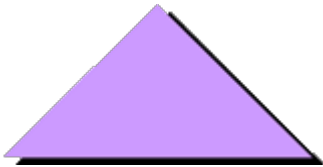
Schweizerische Morbus Crohn / Colitis ulcerosa Vereinigung

ASMCC

Association Suisse de la Maladie de Crohn et Colite ulcéreuse



SVOI ASOI



DRAVET

Association Syndrome de Dravet Suisse
Vereinigung Dravet Syndrom Schweiz
Associazione Sindrome di Dravet Svizzera



CUORE MATTO

debra
because the cost of doing nothing is too great

fabrysuisse



**FÖRDERVEIN
FÜR KINDER MIT
SELTENEN KRANKHEITEN**

47 ❄️ ❄️ ❄️

Verein
Klinefelter-Syndrom
Schweiz



lysosuisse

lysosomale speicherkrankheiten schweiz
maladies lysosomales suisse
malattie lisosomali svizzera



**Morbus
Wilson**

NPSUISSE

Schweizerische Niemann-Pick Vereinigung
Association Suisse Niemann-Pick
Associazione Svizzera Niemann-Pick
Associazione Svizzera Niemann-Pick
Swiss Niemann-Pick Association



3 Rare Disease Day, February 2015



4 Federal Act on Research involving Human Beings (Human Research Act, HRA)

Art. 16 Informed consent

- 1 Persons may only be involved in a research project if they have given their informed consent. Consent must be given in writing; the Federal Council may specify exemptions.
- 2 The persons concerned must receive comprehensible oral and written information on:
 - the nature, purpose and duration of, and procedure for, the research project;
 - the foreseeable risks and burdens;
 - the expected benefits of the research project, in particular for themselves or for other people;
 - the measures taken to protect the personal data collected;
 - their rights.

4 Federal Act on Research involving Human Beings (Human Research Act, HRA)

Art. 19 Liability

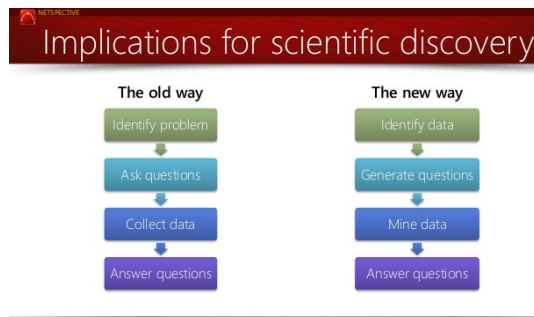
- 1 Any person who carries out a research project involving persons shall be liable for damage suffered by them in connection with the project. The Federal Council may specify exemptions from liability.
- 2 Compensation claims become time-barred three years after the injured party has become aware of the damage and of the liable party, but no later than ten years after the completion of the research project. The Federal Council may specify a longer limitation period for particular research areas.
- 3 The provisions of the Code of Obligations⁴ on tort are otherwise applicable; in the exercise of official duties, the Government Liability Act of 14 March 1958⁵, or cantonal government liability law, is applicable.

Care4brittlebones.org

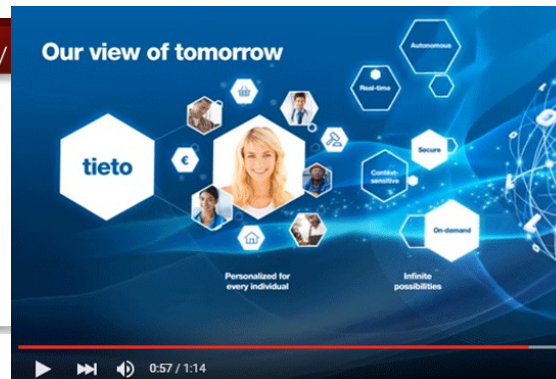


Care4brittle bones: A strategic approach to improve OI...until we find a cure!

DATA



STRATEGY



CULTURE



Open Source Document
Patient Outcome metrics

Research App (Radboud
University)

Strategy Workshop of
Experts&OI people

Research Networks for 3
key disciplines

6 Patient involvement: becoming an active partner in research

– „Research subject“

Partner in research

Information provider

Advisor

Reviewer

Co-researcher

- Driving force

6 Patient involvement: professional perspective

- Representativeness
- Quality
- Bias
- Influence
- Consumer expectations,
- Cost increase and research duration
- Roles overlapping

Consumer involvement in health research: a review and research agenda; Jonathan Boote*, et. al. 2002

7 Concluding discussion

- Is the Swiss health system ready for active patient involvement?
- Can Swiss patient organisations become active partners in research?
- Patient organisations as active partners: what do we need to lobby for or to do to get there?

- What is your experience in involving **patient partner organisations** in your research projects?

Bibliography: see annexe

The image shows a screenshot of the bibme website interface. At the top, there is a dark blue navigation bar with the bibme logo and a Chegg service tag on the left, and links for 'My Bibliographies', 'Plagiarism Checker', 'Citation Guide', 'Title Page', 'Support', 'Log In', and 'Register' on the right. Below this, there are three buttons for citation styles: 'APA' (highlighted in blue), 'MLA', and 'Chicago/Turabian'. A horizontal bar below these buttons contains five categories: 'Journal', 'Website', 'Book', 'Video', and 'Other'. The 'Book' category is currently selected, indicated by a white arrow pointing to it. Below this bar is a search input field with the placeholder text 'Find a book by title, author...' and a yellow 'Search Book' button. To the left of the search field, there is a link for 'Auto-fill mode'. Below the search field, there is a section titled 'Your Bibliography' with the text 'Any citations...' and two buttons for 'APA' and 'MLA'. To the right of these buttons, there is a line of text: '...need, you can edit or delete any of your citations.' Below this section, there is a 'Help' section with two buttons for 'Journal' and 'Website'. A large black circle is drawn around the 'Journal' and 'Website' buttons in the 'Help' section, with a white mouse cursor arrow pointing to the 'Journal' button. The bottom of the page features a blue navigation menu with a home icon and a blue circular button.



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Thank you for your attention.

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Special thanks

Céline Moret, Retina Suisse

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Allianz Seltener Krankheiten - Schweiz
Alleanza Malattie Rare - Svizzera



ASRIMM

Association Romande du Syndrome de Sjögren



BLACKSWAN FOUNDATION

SWISS FOUNDATION FOR RESEARCH ON CYPRIAN DISEASES
SCHWEIZERISCHE STIFTUNG FÜR DIE FÖRDERUNG SÜDBERNER KRANKHEITEN
FONDATION SUISSE POUR LA RECHERCHE SUR LES MALADIES CYPRIENNES
FONDAZIONE SVIZZERA PER LA RICERCA SULLA MALATTIA CIPRIANA



FONDATION FILIPPO Suisse



Association STB Suisse



Sclérose tubéreuse de Bourneville
Tuberöse Sklerose Komplex
Sclerosi tuberosa complessa



Xtraordinaire.org

Handicaps mentaux liés au chromosome X



Die, Monex...
Je suis Xtraordinaire, c'est ça ?

Partageons nos expériences pour mieux les aider



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Schweizerische Hämophilie-Gesellschaft
Association Suisse des Hémophiles
Società Svizzera Emofilia

