SOCIAL REVOLUTION WORKSHOP SESSION I

SMALL GROUP DISCUSSIONS

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EURORDIS Social Policy Advisory Group

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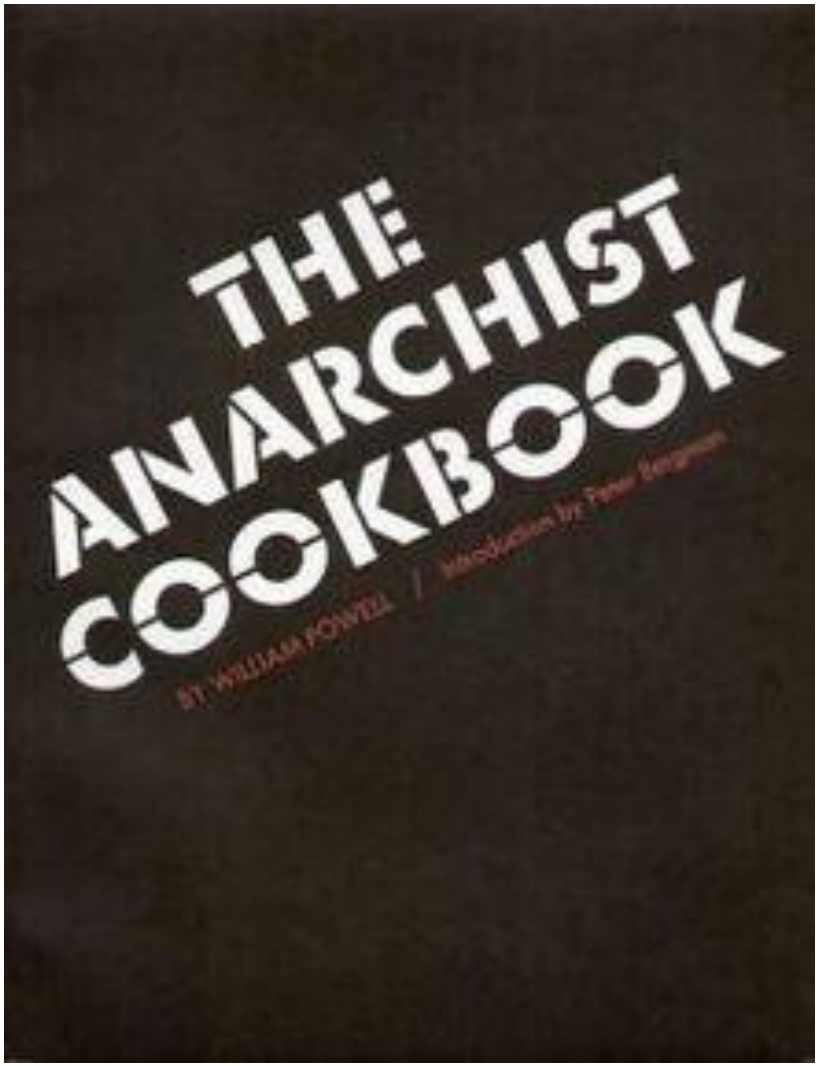
THE
ANARCHIST
COOKBOOK

A EURORDIS INITIATIVE

INNOVCare

9.35 - 20'	**Impact on daily life and related need**	*Sandra Courbier*
9:55 - 15'	**Discussion**	*In groups*

10.10 - 10'	**Impact on well-being and mental health**	*Erwan Berjonneau*
10.20 - 15'	**Discussion**	*In groups*

10:35 - 25'	**Recap from group discussions**	*1 person/group*

11.00 – 30'	**Coffee break**	

11.30 - 15'	**The challenge of coordination of care**	*Raquel Castro*
11.45 - 15'	**Discussion**	*In groups*

12.00 - 15'	**Challenges of working while living with a rare disease**	*Sandra Courbier*
12.15 - 15'	**Discussion**	*In groups*

12.30 - 25'	**Recap** **Instructions for voting**	*1 person/group* *Chair*
12.55 - 5'	**Voting**	*Individually*

What do we need to ask for based on these results? *(at least 1; no more than 3 per chapter)*

Actions that we can ask for/do? Policy proposals we can push for?

National level
European level

They can be recommendations to:

- Patient organisations
- National authorities
- European Institutions
- Social Services
- General recommendations for various stakeholders

What priorities?

Voting rules

- use all your stickers (4 in total)
- 1 sticker next to 1 action/recommendation
- you can put multiple stickers on 1 flip over sheet

What will happen with your votes?

- counted and the top-3 will be fed back during the plenary session

Thank you !

See you at 14 hours at the afternoon session!

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RARE DISEASES EUROPE

Examples of EURORDIS position papers

- Early access to medicines in Europe: Compassionate use to become a reality (April 2017)

Policy proposals

EURORDIS proposes one of the following options:

1. **Promote the French ATU system** so that every Member State adopts it, as it is probably the most efficient compassionate use scheme; or

2. **Adopt European legislative measures** which would confer a greater role in the organisation of CUPs upon the EMA; and/or

3. **Apply the Directive on Patients' Rights in Cross-Border Healthcare** to include compassionate use as part of the care basket so that patients can benefit from these treatments wherever they live in the EU; and/or

4. **Apply Medicines Adaptive Pathways to Patients to all medicines**, where the EU regulator may authorise a medicine at an early stage for a limited group of patients who have a great need for the product, keeping in mind that post-authorisation confirmatory studies need to be conducted afterwards. This is in the spirit of the compassionate-use programme as defined by the EU legislation, but with a different regulatory angle. This can only work if payers are part of the initiative, as they will need to accept to pay for a medicine with high uncertainties in term of efficacy or safety at that point; and

Inequalities in accessing compassionate use medicines were presented to the European Medicines Agency back in 1998. These inequalities undermine the success of the European legislation on pharmaceuticals.

5. **Amend the EMA guidelines for compassionate use** so that the role of the EMA could be reinforced.

Recommendations

To Member States

- National authorities should improve the transparency of the compassionate use programmes they authorise, so that clinicians and patients are aware of which programmes are run in which countries and how to join them
- Member States should create a compassionate use programme Facilitation Group in order to exchange information and build upon common experiences to set up harmonised procedures and create a network which can facilitate future changes in the legislation
- Member States should respect article 83 of Regulation (EC) Nº 726/2004 and notify the EMA of compassionate-use programmes that they authorise.

To European authorities

- The European Commission could compare different national schemes for compassionate-use programmes available in the EU
- The EMA could explore how to make better use of the European register of clinical trials to identify clinical trials whose purpose is to provide a medicine on a compassionate basis (typically open-label trials with no comparison arm).