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Involving Stakeholders in the Assessment of Screening Policies:

A deliberative experience on Newborn Screening for Cystic Fibrosis

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Plan

- Creating a deliberative forum
- Planning a Cystic Fibrosis forum
- Organising the Cystic Fibrosis FORUM
- Results
- Conclusion

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Creating a deliberative forum (1)

- The INSPQ was mandated by the Québec Ministry of Health and Social Services (MSSS) to "*document and clarify key issues and propose appropriate options related to the diagnostic (including screening) and initial follow-up of newborns affected by cystic fibrosis in Quebec*".
- Screening programs are **complex** in nature
- Decision-making usually takes place in a context of **uncertainty**, exacerbated by **lack** or **limited quality** and **validated data**

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Creating a deliberative forum (2)

- Necessity of a scoping review
- Taking into account the Québec clinical and organisationnal context
- Looking at the best practices from other HTA organisations
- Discussions with John Lavis about the McMaster Health Forum

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Creating a deliberative forum (3)

Involvement of stakeholders in order to :

- deliberate on **conflicting evidence interpretations**;
- produce **new contextual data**; and
- identify emerging **consensus** on important problems and potential solutions.

But also to:

- increase **transparency**, **accountability** and **legitimacy** of an advisory process;
- consider stakeholders **values**, **needs** and **knowledge;**
- help decision-making regarding **social and ethical dilemmas**; and
- maintain decisions' **relevance and acceptability.**

(Gauvin, F.P. La délibération pour éclairer la prise de décision: L'expérience du McMaster Health Forum. Presentation)

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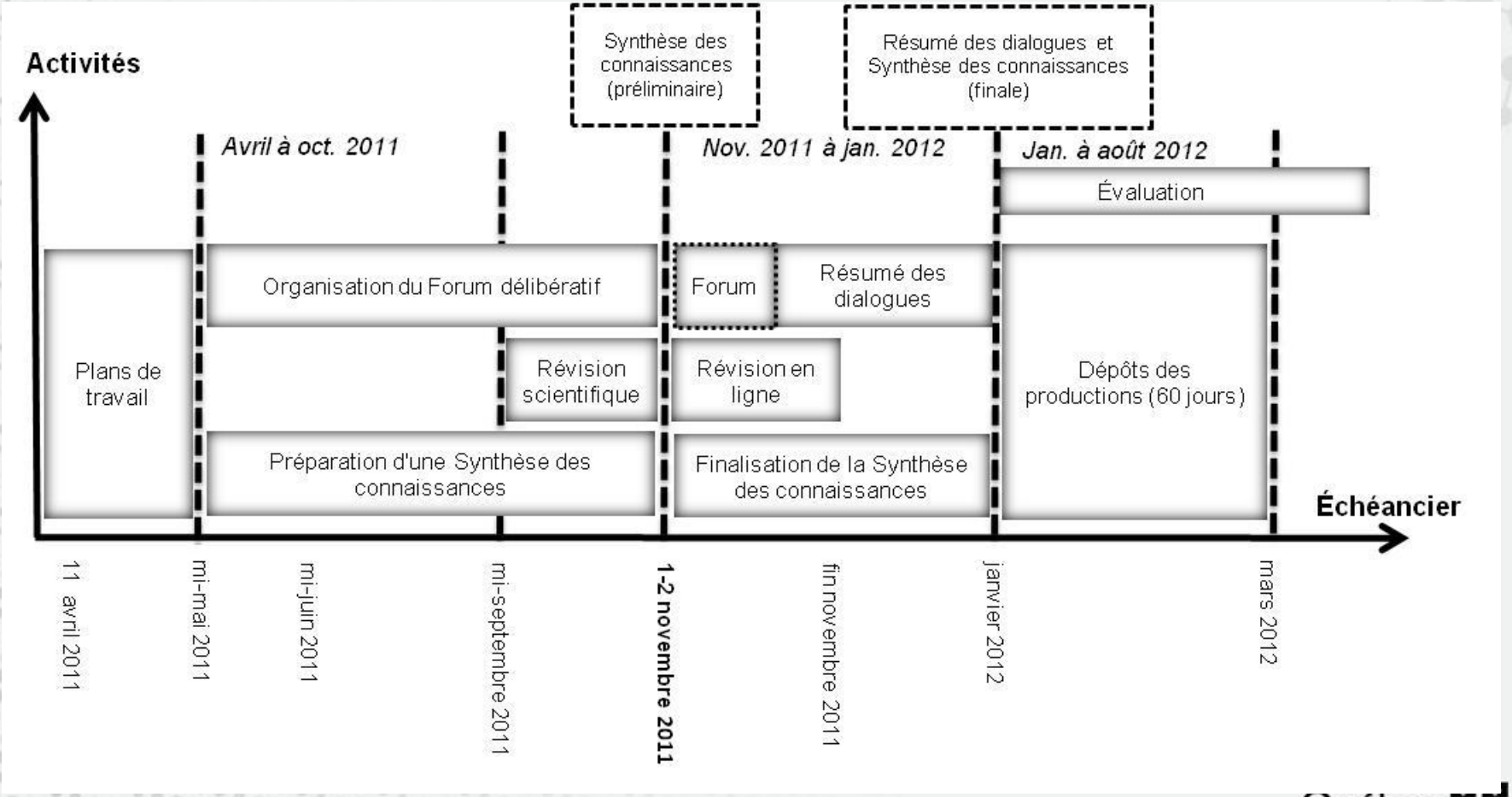
Planning a cystic fibrosis forum (1)



Québec

Planning a cystic fibrosis forum (2)

1. Review current evidence and litterature on the matter;
2. Document the Quebec-specific context using qualitative methods;
3. Invite and interview potential key stakeholders (i.e. representatives, experts, users, patients, decision-makers, etc.);
4. Share current knowledge on the issues with participants;
5. Create a two-day event to deliberate on specific issues;
6. Supplement current knowledge with any new contextual information and resume dialogues into a second coherent document; and
7. List and discuss key diagnostic, newborn screening and treatment options.

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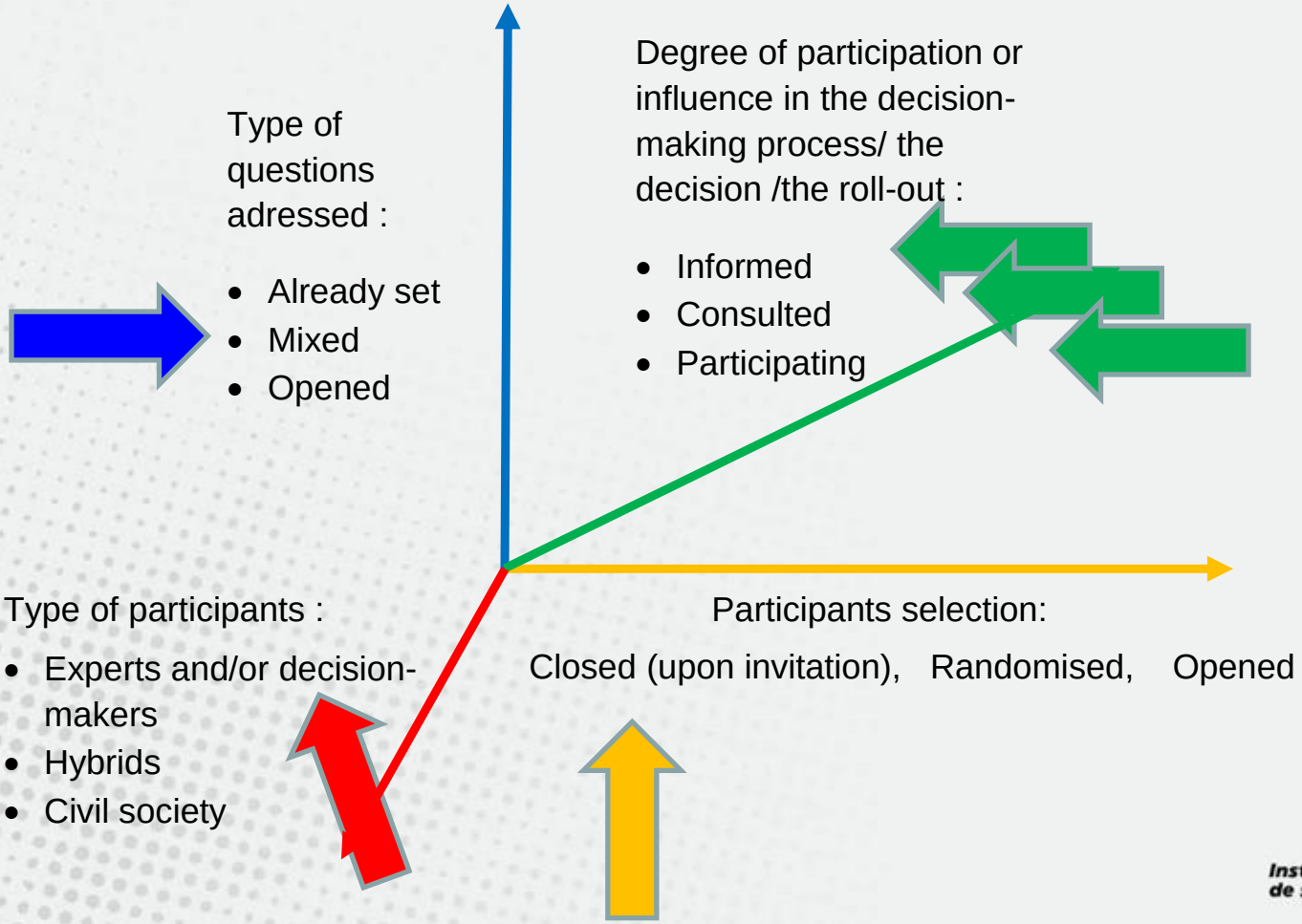
Planning a cystic fibrosis forum (3)



Organising the Cystic Fibrosis FORUM (1)

1 year process (May 2011 -> May 2012)

Three deliverables:

- A review of the litterature hereafter named **Evidence review**
- A two-day event hereafter named the **Cystic Fibrosis Forum**

- A deliberation summary hereafter named the **Dialogue summary**

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Organising the Cystic Fibrosis FORUM (2) Developing the Evidence review

Objectives

- Summarize **current knowledge** on :
  - clinical and genetic aspects, the condition;
  - morbidity, prognosis and mortality; and
  - CF screening, diagnostic, treatment and initial follow-up.
- Document the **context** of Québec's specialized CF centers and laboratories
- Highlight current **issues** related to CF screening, diagnosis (as well as misdiagnosis), treatment and initial follow-up
- Address **potential options**

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Organising the Cystic Fibrosis FORUM (3) Developing the Evidence review

Methodologies and data sources:

- Review of reviews
- Two bibliographic research strategies (general, Québec/Canada centered)
- Cystic fibrosis Canada's registry
- Interviews with key informants
- CF clinics questionnaires
- Scientific review : internal, external, online
- Disclosure of potential conflicts of interest

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Organising the Cystic Fibrosis FORUM (4) Selecting participants

- Preliminary interviews (30) with potential participants to determine:
  - Interest towards the subject and deliberation
  - Potential contributions to the forum
  - Issues that participants would like to see adressed
- Maintain representativeness and balance between viewpoints: Public health; Citizens and patients; Clinical; Management and laboratory; Ethical and legal; Methodological and research; Economical; Organizational
- Select 17 individuals

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Organising the Cystic Fibrosis FORUM (5)
A 2-day event

- Event's program
- Event's presentations (3 guest speakers)
- Chatham House rules (*"When a meeting, or part thereof, is held under the Chatham House Rule, participants are free to use the information received, but neither the identity nor the affiliation of the speaker(s), nor that of any other participant, may be revealed".)*
- Deliberation moderators (reference tools, power point presentations)
- Event's logistics (audio files, filmed interviews, conference room, booking, food, etc.)

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A moderator

17 participants

Observers

Three presentations

From left to right: Yves Giguère, David Simard, Andréa Ruchon, Valérie Borde, Clara Popa, Donald Aubin, Jacques-Édouard Marcotte, Pierre-Alexandre Tremblay, Joe T. R. Clarke, Patrick Daigneault, Julie Saucier, Valérie Marchand, Robert Jacob, Karine Sénécal, Larry Lands, Rachelle Laframboise, Jocelyne Cousineau

Results (1)

A better understanding of :

- Misdiagnosis' causes for newborns in Québec;
- Delays from CF diagnosis (biochemical tests, DNA tests, sequencing) and the initiation of treatments;
- Problems related to diagnostic tests (sweat test: standardization, normalization and regulation);
- Existence of other non validated diagnostic tests.

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Results (2)

Unanswered questions:

- The newborn screening protocol performance;
- The organisation of newborn screening in other countries and jurisdictions;
- The cost-effectiveness and budget impact of a potential newborn screening program;
- The management of a number of false positives and false negatives (very low according for those currently tested);
- The management of CFTR related conditions generated.

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Results (3)

Unanswered questions:

- Coverage for individual from ethnic groups with DNA panels (screening test);
- CFTR gene sequencing outside Québec;
- Drug, technologies and medical services coverage by public and private insurance regimes;
- Explicit newborn screening consent;
- Identification of the carrier status and the information depository; and
- Organisation of genetic counselling.

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Results (4)
Developing the Dialogues' Summary

Objectives:

- Translate Forum's discussions into a coherent narrative; and
- Highlight new contextual issues, clarify current clinical practices, relate converging ideas and unanswered questions.

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Conclusion

- Improved results and conclusions with a contextualized evidence review
- Enthusiasms of stakeholders in participating to a well-organised FORUM
- Great interest in the forthcoming steps and further document key issues, benefits, risks and costs discussed throughout the Forum
- Proposals to the MSSS for further work on some specific issues identified such as …

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