# Use of RWE in Decision Making: Now & in the Future

**Janssen Canada**

CAPT-ACTP 2018 Conference

*Taking Action on Real World Evidence: From Analysis to Impact*

Jennifer Jacobs, *Stowaway*

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# Use of RWE in Health Care Decision Making
## Now and in the Future

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**Example #1**



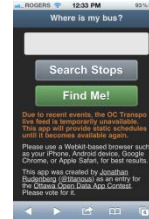City of Ottawa releases bus GPS data, holds contest to stimulate innovation

**2010**

Teen, Jonathan Rudenberg, develops "Wher is my Bus?" app (Ali Ali-Munayer wins silver for "My OC Transpo/BusBuddy" app)

**2011**

OC Transpo realizes it could develop its own app, and raise $1.1 million in revenues.

Stops sharing data.

**2012**

OC Transpo releases "My Transit" app.

Public outcry.

Data re-released

**2018**

GPS data has some shortcoming.

Private, Montreal-based company announces "Transit" app with crowdsource feature.
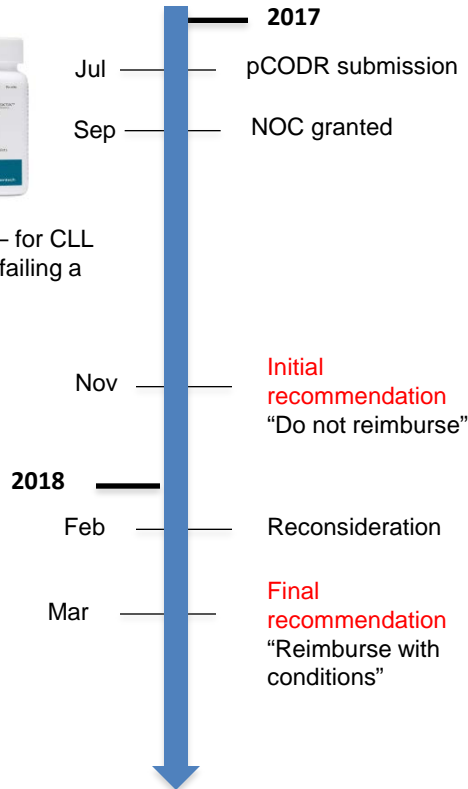
**Transit**

★★★★⯪

**BusBuddy Ottawa**

★★★★☆

**Route 613**

★★★★⯪

**MyTransit**

★★☆☆☆

**Example #2**



**2017**

Jul — pCODR submission

Sep — NOC granted

Venclexta – for CLL
in patients failing a
BCRi

Nov — Initial
recommendation
"Do not reimburse"

**2018**

Feb — Reconsideration

Mar — Final
recommendation
"Reimburse with
conditions"

pCODR systematic review identified one clinical, two *contextual,* studies
1) prospective, open-label cohort study (M14-032)
2) retrospective registry study (Mato , 2016)
3) retrospective registry study (Mato , 2017)

Initial
*"pERC noted the results of the Mato et al. studies, which were retrospective analyses of patients relevant to the current reimbursement request. The results of these analyses, though promising, are prone to several forms of bias and must be interpreted with caution. "*

Final
*"Upon reconsideration of the pERC Initial Recommendation, pERC noted feedback from stakeholders regarding the results from the Mato et al. publications."*

*"Following a lengthy discussion on these factors, pERC was swayed by the totality of evidence and feedback received from stakeholders and agreed that there may be a net overall clinical benefit with venetoclax in this population. pERC however stressed that considerable uncertainty remains in the magnitude of benefit with venetoclax. "*

**Example #3**

First application
CDR systematic review identified two RCTs
1)(TRAFFIC [N = 559] and TRANSPORT [N = 563])

*"no RCTs that evaluated the efficacy of LUM/IVA … in patients younger than 12 years. "*
*"no RCTs designed to examine the effect of LUMA/IVA treatment on …long term disease progression…mortality"*

Second application
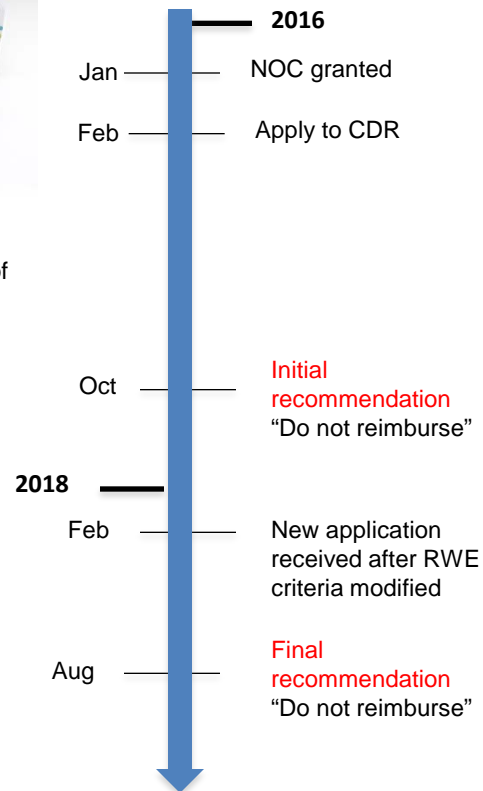CDR systematic review identified four RCTs
1)(TRAFFIC [N = 559] and TRANSPORT [N = 563]; Study 112; Study 109) and one pivotal, single-arm, open-label trial (Study 11B)

*To address long-term disease progression, the manufacturer conducted a match-adjusted indirect comparison*

*"Due to limitations in the analysis, concerns regarding the comparability of the patients from the clinical trials and those from the registry, and issues regarding the generalizability of US registry patients with Canadian patients with CF, it is uncertain if treatment with L400/IVA would have a similar impact on the rate of lung function decline in Canadian patients.. "*

Orkambi – for CF patients > 6 years of age F508del homozygous

**2016**
Jan — NOC granted
Feb — Apply to CDR

Oct — Initial recommendation "Do not reimburse"

**2018**
Feb — New application received after RWE criteria modified

Aug — Final recommendation "Do not reimburse"

**Issues**

| Original data holders | | Data custodians | | Analysis | | Policy | | Impact |

What obligations for those who generate data?

- To share?
- To capture socially-relevant data?

What obligations for those who package data?

- To use common data structures?
- To share?

What obligations for those who analyse data?

- To publish findings?
- To use similar / standard methods?
- To share underlying data?

What obligations for those who interpret data for policy? (i.e., HTA)

- To use similar / standard approaches?
- To conduct original analyses ?

# What is the RWE landscape?

**Holders:**

- CAPCA
- MoHs
- Registries
- Private sector
    - Physicians
    - Labs

**Custodians**
- Public sector
    - Health Canada
    - CIHI
    - Provinces
- Private sector
    - IQVIA
    - EMR vendors
    - QI vendors

**Analysts**
- Public sector
    - e.g., ICES scientists
    - PMPRB
- Private sector
    - Industry
    - CROs

**Interpreters**
- Public sector
    - Health Canada
    - CADTH / PMPRB
    - PAAB

**Pan-Canadian Initiatives**
- HC/R2D2        - CADTH
- PAAB            - DSEN
- CAPCA          - Others?

# Questions for consideration

- Who should analyze data?
  - Only data custodians? data holders?
  - Private sector/public sector or both?
  - Can we share? How do we share?

- How should we interpret real-world data?
  - What do we do with private data/interpretation?
  - How do we move forward in decision-making once standards are developed for interpretation of RWE?
  - What is the process for engagement among various stakeholders around interpretation and utilization of RWE to inform decisions?
  - Is this different from RCTs?
  - Can we use non-Canadian RWE?

- How can we work together around RWE used for decision-making?
  - How do we work together in a transparent way to bring forward the best approach for using RWE to ensure patients get timely access to medicines?

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