

New Horizons GIST 2017 Real World Data



Real World Data/Evidence

For the management of cancer care

For the decision making that is not derived from
randomized clinical trials

Since the creation of the LRG in 2000, cancer diagnosis and treatment has changed dramatically

- **Molecular testing** expansion (more widespread and cheaper costs) has created more treatment targets
- Targeted *oral drugs* have replaced many non-targeted *IV drugs*
- **Patient Survival** has increased for many cancers, including GIST

As a Consequence

- Cancer sub-groups have increased, including for rare cancers
- Smaller data sets
 - Clinical trials more difficult
 - Drug Development harder
 - Drugs more expensive
- More patients can be treated by local oncologists-lacking expertise/experience
- Patients are required to take oral drugs daily for prolonged periods of time> non-compliance
- Long term side effect monitoring and management, including new ones, is required

In contrast, cancer research structures and culture remain stuck in time

- Traditional randomized clinical trials cannot provide all the data/evidence needed to manage cancer care in a timely way
- Data/evidence sharing remains bogged down in a post publication structure that delays access for months to years
- Clinical trial and research failures often go unreported denying access to crucial information about what not to do
- Individual Researcher competition remains a reality versus collaboration
- This is compounded by pharma competition whose market priority often remains a reality over what is required for cancer patient survival such as the combination of drugs owned by separate companies

*Lethal time gaps continue to hamper cancer patient survival
and well-being*

As a consequence, real world data/evidence is needed to supplement that provided by formal randomized clinical trials

What is real world data/evidence?

Other than a general consensus that it is not that which is provided by formal randomized clinical trials the term means different things to different people and thus becomes rather nebulous.

How should we evaluate real world data/evidence?

It should facilitate decision making needed by clinicians and patients/care-givers for cancer patient survival and well-being

And it should be

- Accurate: Including rigorous quality controls
- Timely: Reducing the lethal time lag between discovery and publication
- Reflective of the patient's perspective about treatment efficacy and side effects
- Across the continuum of patient care from initial diagnosis to end of life across institutional barriers
- Accessible: Including to the patient/care-giver without cost
- Portable: As a patient moves to a new trial or treatment site
- A support for clinical trial recruitment
- A source of health economic data for comparative effectiveness research

Sources of real world data/evidence

- **The Life Raft Group data/evidence platforms:**
 - **Patient Registry and Tissue Bank (patient powered)**
 - **GIST Prime™ (patient powered)**
 - **SideEQ (patient powered)**
 - **InterGR™ - under development**
 - **Surveillance-under development**

Real World Evidence on a Global Scale

- Cancer Survival: The Start of Global Surveillance
 - A large international cancer survival study called CONCORD-2 was published online in November 2014 in the medical journal *The Lancet*.
 - The [CONCORD Programme](#) is led by the Cancer Research UK Cancer Survival Group at the London School of Hygiene and Tropical Medicine in London, United Kingdom.
 - The goal of the study was to produce estimates of cancer survival across many countries that can be compared so scientists can begin to determine why survival differs among countries.
 - The study reports trends in five-year survival between 1995 and 2009 for close to 26 million cancer patients diagnosed with one of 10 common cancers: breast (women only), cervix, colon, leukemia, liver, lung, ovary, prostate, rectum, and stomach.

Five-Year Survival Rates for Patients Diagnosed with Five Common Cancers in Seven Countries

2005–2009

| Country | Breast | Colon | Lung | Prostate | Childhood Leukemia |
|-----------------|--------|-------|------|----------|--------------------|
| Canada* | 85.8 | 62.8 | 17.3 | 91.7 | 90.6 |
| France** | 86.9 | 59.8 | 13.6 | 90.5 | 89.2 |
| Germany | 85.3 | 64.6 | 16.2 | 91.2 | 91.8 |
| Italy | 86.2 | 63.2 | 14.7 | 89.7 | 87.7 |
| Japan | 84.7 | 64.4 | 30.1 | 86.8 | 81.1 |
| United Kingdom* | 81.1 | 53.8 | 9.6 | 83.2 | 89.1 |
| United States | 88.6 | 64.7 | 18.7 | 97.2 | 87.7 |

*Countries with 100% population coverage.

**100% population coverage for children only.

Under-five mortality from the World Health Organization

| Rank | Country | Under-five mortality rate |
|------|-----------------------|---------------------------|
| 2 | <u>Mexico</u> | 13.2 |
| 5 | <u>United States</u> | 6.5 |
| 8 | <u>Poland</u> | 5.2 |
| 9 | <u>Canada</u> | 4.9 |
| 11 | <u>France</u> | 4.3 |
| 12 | <u>United Kingdom</u> | 4.2 |
| 16 | <u>Switzerland</u> | 3.9 |
| 18 | <u>Netherlands</u> | 3.8 |
| 19 | <u>Germany</u> | 3.7 |
| 22 | <u>Italy</u> | 3.5 |
| 24 | <u>Austria</u> | 3.5 |
| 26 | <u>South Korea</u> | 3.4 |
| 29 | <u>Japan</u> | 2.7 |
| 34 | <u>Luxembourg</u> | 1.9 |