**Supplementary File 1.**

**RWE Asia Needs Assessment Survey**

# About this survey

There is growing interest in the use real world data (RWD) and real-world evidence (RWE) for regulatory and reimbursement decision making. With the aim of developing a guidance document on RWE for the Asian region, the Saw Swee Hock School of Public Health in the National University of Singapore and the Health Intervention and Technology Assessment Program (HITAP) from Thailand initiated the establishment of an RWD and RWE working group with HTAsiaLink members to further our understanding of optimal use of RWD and RWE for HTA reimbursement decision making. In preparation for the meeting on 27 Apr 2019, we would like to conduct a needs assessment.

Notes:

1. By HTA agency, we are referring to the agency that does the HTA analysis, not the decision making body.
2. By real world data (RWD) and real world evidence (RWE), we will focus on clinical effectiveness data from non-experimental studies (e.g. registries, claim databases, observational studies). That is, not including cost and quality of life assessment.
3. By end users, we refer to researchers, clinicians, industry, etc.

# Instructions for completing the survey

We will appreciate if you take some time to complete this survey and return your responses by 26 April 2019 (Fri). This survey is divided into three sections and will take an estimated 15min of your time. Section 1 collects background information about you. Section 2 asks about existing work in RWD/RWE. Section 3 asks about your interest in some of the future events we have planned. The questions are multiple-choice in nature with space for additional information. Your responses will be confidential. We will treat that the opinions expressed are your own and does not represent your HTA agency.

If you have any question before participating in the survey, please email Dr Hwee-Lin WEE at [weehweelin@nus.edu.sg](mailto:weehweelin@nus.edu.sg)

# Section 1 – Background information

1. Basic demographics:
2. Country you are working in on HTA
3. Organisation [Optional]:
4. Your role:

* HTA decision maker
* Senior management in HTA agency
* Analyst in HTA agency
* HTA researcher in academia
* Clinician
* Others, please specify: \_\_\_\_\_\_\_\_\_\_\_\_

1. Do you have any personal experience with clinical effectiveness data from non-experimental studies? Select all that apply.

* Yes, I collect them
* Yes, I analyse them
* Yes, I evaluate them
* No

3a) If yes: Are any of these experiences related to HTA.

* Yes
* No

# Section 2 – On the use of clinical effectiveness data from non-experimental studies

1. Does your HTA agency have an existing guidance document for end users on the circumstances under which clinical effectiveness data from non-experimental studies are mandatory?
   1. Yes
      1. If yes,
         * when was this guidance released?

Date: \_\_\_\_\_\_\_\_\_\_

* + - * is the guidance available in English?
        1. Yes
        2. No
      * is the guidance publicly available?
        1. Yes
        2. No.

If no, is the guidance available on request?

Yes

No

* + - * Who has previously created this guidance document?

Name of organization: \_\_\_\_\_\_\_\_\_\_\_\_

* 1. No

1. Does your HTA agency have an existing guidance document for clinicians or industry on the minimum standards for collecting and submitting clinical effectiveness data from non-experimental studies for HTA in reimbursement decision making?
   1. Yes
      1. If yes,
         * when was this guidance released?

Date: \_\_\_\_\_\_\_\_\_\_\_\_

* + - * is the guidance available in English?
        1. Yes
        2. No
      * Is the guidance publicly available?
        1. Yes
        2. No.

If no, is the document available on request?

Yes

No

* 1. No

1. Does your HTA agency currently accept clinical effectiveness data (e.g. relative risk, odds ratio, sensitivity and specificity, etc) from non-experimental studies (e.g. registries, claim databases, observational studies) for HTA in informing or making reimbursement decisions?
   1. Yes
      1. If yes, what patient population do the data come from? Select all that apply.
         * Rare disease
           1. Select all that apply:

Phenylketonuria

Thalassemia

Osteogenesis Imperfecta

Mucopolysaccharidoses

Spinal Muscular Atrophy

Other rare disease, please specify: \_\_\_\_\_\_\_\_\_\_\_\_\_

* + - * Cancer
        1. Select all that apply:

Advanced colorectal cancer

Advanced breast cancer

Advanced non-small cell lung cancer

Other cancer, please specify: \_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

* + - 1. Immunology
      2. Type 2 diabetes mellitus
      3. Others, please specify: \_\_\_\_\_\_\_\_\_\_\_\_\_
    1. Do you require people who submit HTA dossiers to provide justification(s) for the use of clinical effectiveness data from non-experimental studies?
       1. Yes
       2. No
    2. How much confidence in making a reimbursement decision do you personally have in the clinical effectiveness data from non-experimental studies submitted?
       1. Very high confidence
       2. High confidence
       3. Moderate confidence
       4. Little confidence
       5. No confidence
  1. No
     1. If no, why? Select all that apply:

We think that non-experimental data should not be used for HTA at all

We do not have the technical expertise to review the non-experimental data

Manufacturer is not willing to collect non-experimental data

Others, please specify: \_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

1. Does your HTA agency provide specific guidance on how to account for confounding factors when analysing RWD?
   1. Yes
      1. If yes, would you be willing to share the guidance document with us?
         * Yes
         * No
   2. No
2. Does your HTA agency provide specific guidance on how to reduce selection bias when designing a non-experimental study?
   1. Yes
      1. If yes, would you be willing to share the guidance document with us?
         * Yes
         * No
   2. No
3. Has your HTA agency encountered situations where randomized clinical trial (RCT) data are not available and you have made reimbursement decisions solely on clinical effectiveness data from non-experimental studies?
   1. Yes
      1. If yes, will you please provide more information? e.g. what is the patient population? What was the justification provided by manufacturer for not conducting RCT? \_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_
   2. No
4. Does your HTA agency currently accept clinical effectiveness data from pragmatic clinical trials?
   1. Yes
   2. No
      1. If not, are you planning to?
         * Yes, within the next 6 months
         * Yes, but not so soon
         * No
5. Does your HTA agency think that pragmatic clinical trial should be conducted for every novel therapy to be considered for reimbursement?
   1. Yes
      1. Would you like to share with us more on why pragmatic clinical trials should always be conducted?
   2. No
      1. Would you like to share with us more on when pragmatic clinical trials should be conducted?
6. What are the challenges that your HTA agency encounters with regards to clinical effectiveness data from non-experimental studies? Select all that apply.
   * The patients were not followed up over a sufficient period of time
   * There was not enough evidence in the HTA dossier that the patient sample selected truly reflect the patients in routine clinical care
   * The patient sample in the HTA dossier was too similar in characteristics with patients who took part in the clinical trials that led to regulatory approval
   * The HTA dossier included outcomes data that are not relevant
   * The HTA dossier did not clearly report other treatments that the patients are receiving
   * The HTA dossier did not clearly report other comorbidities that the patients have
   * The HTA dossier included data on a patient sample that does not reflect the patients in routine clinical care
   * We do not have the expertise to evaluate if confounding factors are properly accounted for
   * Others, please specify: \_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_
7. Has your HTA agency ever requested HTA dossiers to include clinical effectiveness data from non-experimental studies at doses that are lower than those stated on drug labels?
   1. Yes, because we believe that Asians have lower body weight and can use lower dose
   2. Yes, because we have received reports on adverse drug reactions
   3. Yes, other reasons, please specify: \_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_
   4. No, because this is the role of our regulatory agency
   5. No, this has not crossed our mind
   6. No, other reasons, please specify: \_\_\_\_\_\_\_\_\_\_\_\_\_\_
8. Besides clinical effectiveness, has your HTA agency ever requested for or used any of the following data from non-experimental studies? Select all that apply.
   * Disease prevalence
   * Long-term safety data
   * Health utilities
   * Medication adherence
   * Transition probabilities between health states in economic model / Rate of disease progression
   * Others, please specify: \_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_
9. What is the single most important problem related to the use of clinical effectiveness from non-experimental studies in HTA that you hope the School of Public Health can address?
10. Do you think this working group should also develop a guidance document on topics other than non-experimental studies? This working group is currently focused on clinical effectiveness. For example,
    * Collection and analysis of cost and health utilities
    * Others, please specify: \_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_