CADTH

Appendix 1: INAHTA Listserv Survey

Listserv topic title: Gene Therapy: International Regulatory and HTA Activities and Reimbursement Status

Agency: CADTH

Project lead and contact information (who should receive the responses): Teo Quay, TeoQ@cadth.ca

Background: Over the last decade, important scientific advances have been made in the field of gene therapy. Recent approvals from regulatory bodies and decision-makers reflect its promise. The US FDA approved the first gene therapy for acute lymphoblastic leukemia (ALL) in children and young adults in August 2017 and the second gene therapy for aggressive lymphoma in adults in October 2017, granting both CAR T-cell technologies Priority Review and Breakthrough Therapy designations. The NHS in the UK announced in October 2017 a decision to fund gene therapy for the treatment of adenosine deaminase deficiency in children at a price tag of over £500,000. In Canada, New Drug Submissions on gene therapy are anticipated in the near future.

To inform CADTH's approach and process for evaluating gene therapy and to help Canadian jurisdictions prepare for this new health technology, an Environmental Scan is being conducted on international regulatory and HTA activities, and reimbursement status on gene therapy. Your responses to the questions below, in addition to a scan of the published and grey literature, will inform this Environmental Scan to help Canadian as well as international jurisdictions plan for gene therapy.

Definition: Gene therapy is defined as "a set of strategies that modify the expression of an individual's genes or repair abnormal genes," involving the administration of a specific nucleic acid (i.e., DNA or RNA) via a viral or non-viral vector. This includes immunotherapy involving genetically-modified T cells (e.g., CAR T-cell therapy) and regenerative medicine involving genetically modified cells or tissues, including stem cells.

Consent: Please note that your response to the survey will be used to prepare a CADTH Environmental Scan Report, which will be available for public access. Your name (and contact information, if provided) will only be used to contact you about your responses to this survey. Your consent does not give CADTH permission to disclose your name within the report.

Please **type in your first and last name** on the line within the consent provided below, to authorize CADTH to use the information provided by you in the Environmental Scan Report.

This information is provided to assist CADTH in conducting an Environmental Scan entitled "Gene Therapy: International regulatory and HTA Activities and Reimbursement Status." By responding to this survey, I First Name Last Name, give my authorization for CADTH to summarize my responses in the published Environmental Scan report and for my organization to be identified as a source for survey respondents. However, I (and the organization I represent) decline any responsibility for the analyses, conclusions, opinions, and statements expressed in CADTH's Environmental Scan Report.

☐ *I agree*

☐ *I do not agree (your responses will be used internally for information purposes only)*

Respondent information:

- **Name (First, Last):**
- **Country:**
- **Region your organization serves (if different from Country):**
- **Email:**

Questions:

The questions below should take you no more than **30 minutes** to complete. Please feel free to add any details or comments to any of the questions. The requested deadline for your responses is **December 22, 2017**.

Regulation

1. In your country, how does the regulator currently categorize CAR T-cell therapy and other gene therapy (please check all answers that apply)?

   a. CAR T-cell therapy

   - ☐ Drug
   - ☐ Biologic
   - ☐ Blood or blood product
   - ☐ Cellular therapy
   - ☐ Gene or genetic therapy
   - ☐ Vaccine
   - ☐ Device
   - ☐ Both drug and device
   - ☐ Other Please explain: _____
   - ☐ Don't know

   b. Other gene therapy

   - ☐ Drug
   - ☐ Biologic
   - ☐ Blood or blood product
   - ☐ Cellular therapy
   - ☐ Gene or genetic therapy
   - ☐ Vaccine
   - ☐ Device
   - ☐ Both drug and device
   - ☐ Other Please explain: _____
   - ☐ Don't know

2. In your country, has the regulator approved any CAR T-cell therapy or other gene therapy?

   a. CAR T-cell therapy

   ☐ No
   ☐ Yes — Please identify which therapies and when they were approved and if possible, attach or provide links to relevant information: _____
   ☐ Don't know

   b. Other gene therapy

   ☐ No
   ☐ Yes — Please identify which therapies and when they were approved and if possible, attach or provide links to relevant information: _____
   ☐ Don't know

HTA (not including reimbursement decisions)

3. In your HTA organization, how are CAR T-cell therapy and other gene therapy currently categorized (please check all answers that apply)?

   a. CAR T-cell therapy

   ☐ Drug
   ☐ Biologic
   ☐ Blood or blood product
   ☐ Cellular therapy
   ☐ Gene or genetic therapy
   ☐ Vaccine
   ☐ Device
   ☐ Both drug and device
   ☐ Other — Please explain: _____
   ☐ Don't know

   b. Other gene therapy

   ☐ Drug
   ☐ Biologic
   ☐ Blood or blood product
   ☐ Cellular therapy
   ☐ Gene or genetic therapy
   ☐ Vaccine
   ☐ Device
   ☐ Both drug and device
   ☐ Other — Please explain: _____
   ☐ Don't know

4. Has your HTA organization produced or is it planning to produce any guidelines or frameworks specifically for evaluating CAR T-cell therapy or other gene therapy?

   a. CAR T-cell therapy

   ☐ No – it is out of scope for us, and we will not evaluate it; if so, please explain why it is out of scope:

   _____

   ☐ No – it is within scope for us, and we will evaluate it using existing process(es); if so, please explain which existing process(es) will be used (e.g., for drugs or devices) and if possible, attach or provide links to relevant information:

   _____

   ☐ Yes – Please explain and if possible, attach or provide links to relevant information:

   _____

   ☐ Don't know

   b. Other gene therapy

   ☐ No – it is out of scope for us, and we will not evaluate it; if so, please explain why it is out of scope:

   _____

   ☐ No – it is within scope for us, and we will evaluate it using existing process(es); if so, please explain which existing process(es) will be used (e.g., for drugs or devices) and if possible, attach or provide links to relevant information:

   _____

   ☐ Yes – Please explain and if possible, attach or provide links to relevant information:

   _____

   ☐ Don't know

Reimbursement

5. In the country/region that your HTA organization serves, have any reimbursement decisions been made regarding CAR T-cell therapy or other gene therapy?

   a. CAR T-cell therapy

   ☐ No

   ☐ Yes – Please identify which therapies and when the decisions were made and if possible, attach or provide links to relevant information:

   _____

   ☐ Don't know

b. Other gene therapy

☐ No – no decisions have been made

☐ Yes – Please identify which therapies and when the decisions were made and if possible, attach or provide links to relevant information:

_____

☐ Don't know

Other Jurisdictions and HTA Bodies

6. In addition to those identified in Questions 1-5, are you aware of any other guidelines or frameworks for evaluating CAR T-cell therapy or other gene therapy or any regulatory, HTA, or reimbursement decisions regarding CAR T-cell therapy or other gene therapy?

a. CAR T-cell therapy

☐ No

☐ Yes – Please explain and if possible, attach or provide links to relevant information:

_____

b. Other gene therapy

☐ No

☐ Yes – Please explain and if possible, attach or provide links to relevant information:

Requested deadline for responses: **December 22, 2017.**

Appendix 2: Information on Survey Respondents

Country	Organization Represented by Survey Respondents
Australia	Adelaide Health Technology Assessment (AHTA)
France	Haute Autorité de Santé (HAS)
Germany	Gemeinsamer Bundesausschuss (The Federal Joint Committee) (G-BA)
Sweden	Statens beredning för medicinsk och social utvärdering (Swedish Agency for Health Technology Assessment and Assessment of Social Services) (SBU) Medical Products Agency (MPA)
Taiwan	Center for Drug Evaluation (CDE)
US	Kaiser Permanente

Note: A respondent from Colombia (Instituto de Evaluación Tecnológica en Salud) provided an incomplete response.