Providing insight into potentially high-impact medicines in the pipeline

- New medicines are constantly reshaping the pharmaceutical landscape, with the potential to significantly impact our healthcare system.
- The PMPRB’s Meds Pipeline Monitor (MPM) provides insight into medicines in the late stages of clinical evaluation that may have significant clinical and financial impacts on drug spending in Canada.
- The 2018 edition of the MPM (previously published under the title New Drug Pipeline Monitor) employs an renewed approach to the selection of pipeline medicines, identifying a comprehensive list of candidates from a wide range of therapeutic areas.
- Its companion report, Meds Entry Watch, tracks newly launched medicines as they enter Canadian and international markets. Together these two PMPRB publications provide decision makers, researchers, patients and other stakeholders with a broad overview of emerging drug therapies in Canada and internationally.
Selecting suitable pipeline candidates

- The selection criteria sorts hundreds of potential candidates through a two-step process that involves an initial screening phase followed by an analytical review phase.

- It focuses on new medicines that may:
  - offer a therapeutic benefit over existing treatments;
  - address previously unmet therapeutic needs; and/or
  - treat serious conditions.

- In addition to the main list of medicines, this edition includes a list of upcoming gene therapies.
1. INITIAL SCREENING

Medicines in phase III clinical trials and pre-registration

- Expected clinical trial end dates within three years of analysis
  - Drug geography includes Canada, U.S. and Europe‡

2. ANALYTICAL REVIEW

- Demonstrates improved safety and/or efficacy
  - Novel mechanism and/or first-in-class
    - One or more of the following:
      1. Breakthrough
      2. Fast Track
      3. Priority Review

- Demonstrates promising safety and/or clinical effectiveness

‡ Only medicines with phase III clinical trials in Canada, United States and/or Europe (excluding Russia and Turkey) are considered as potential candidates.
### Selection criteria* for new medicines

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<th>CRITERIA</th>
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<td><strong>Improved safety and/or efficacy shown in clinical trials</strong>: a medicine that demonstrates increased safety, new outcome measures, increased life expectancy or quality of life</td>
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<td><strong>Novel mechanism / First-in-class</strong>: a medicine that uses a new mechanism of biochemical interaction to produce a medical effect, or a medicine that is the first in its therapeutic class</td>
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<td>In addition, the medicine must fall into one or more of the three following FDA designations for expedited development and review:</td>
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<td>- <strong>Breakthrough</strong> – medicines intended to treat a serious condition and for which preliminary clinical evidence indicates that they may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s)</td>
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<td>- <strong>Fast Track</strong> – medicines used to treat serious conditions and fill an unmet medical need</td>
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<td>- <strong>Priority Review</strong> – medicines that would provide significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications</td>
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*The scientific selection process for gene therapies was less rigorous compared with the other new medicines due to the limited availability of clinical evidence.*
Highlights from the 2018 MPM Pipeline

- Of the 733 new medicinal ingredients in Phase 3 clinical trials and pre-registration, 34 new medicines are featured in this report, including 9 gene therapies.
- The featured list of medicines belong to a total of twelve therapeutic classes, with the oncology class containing the highest number of drugs.

<table>
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<th>Top therapeutic classes</th>
<th>Pipeline drugs</th>
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<td>1. Oncology</td>
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<td>2. Central Nervous System</td>
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- Oncology medicines represent the largest proportion of clinical trials throughout all phases in the pipeline.
### Type of information to be included in the report

<table>
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<th>MEDICINE (TRADE NAME)* COMPANY†</th>
<th>INDICATION</th>
<th>DESCRIPTION</th>
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| Aducanumab Biogen Inc           | Alzheimer's Disease      | • An anti-Amyloid-β(Aβ) monoclonal antibody (immunotherapy) that selectively targets β-amyloid (Aβ) peptide, one of the principal neuropathological markers of the disease.¹  
  • Has demonstrated a favourable safety profile and promising results. It is being studied in early phases of AD to intervene in disease process before it is too late.²  
  • Global revenue forecasted to be $79M in 2019 and $4.98 by 2024.                                    |
| Entinostat Syndax Pharmaceuticals Inc | Metastatic Breast Cancer | • A synthetic benzamide derivative histone deacetylase (HDAC) inhibitor.  
  • Reverses cisplatin resistance.³  
  • Global revenue forecasted to be $16M in 2020 and $423M by 2024.  
  • High-Cost therapeutic area⁴ |
Drug Spotlight
Selected examples of medicines featured in the 2018 MPM report

- **Entinostat**
  - Indicated for metastatic breast cancer
  - Increased efficacy over existing therapies
  - Orphan designation, Breakthrough designation
  - Global revenue forecasted to be $16M in 2020 and $423M by 2024

- **Ubrogepant**
  - Indicated for migraine
  - Shown to have increased safety and efficacy treating acute migraine in a wide range of patients including those who had insufficient response to triptans and patients with moderate to severe cardiovascular risk
  - Global revenue forecasted to be $32M in 2020 and $470M by 2024.

- **Onasemnogene abeparvovec**
  - Indicated for spinal muscular atrophy
  - Promising gene therapy administered as a one-time infusion
  - Global revenue forecasted to be $451M in 2019 and over $2B by 2024
Next Steps:
- Expected release: May 2019
- Sign up for information session, please contact: pmprb.npduis-sniump.cepmb@pmprb-cepmb.gc.ca