# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION
**Washington, D.C. 20549**

## FORM 8-K

**CURRENT REPORT**
**Pursuant to Section 13 or 15(d)**
**of The Securities Exchange Act of 1934**

**Date of Report (Date of Earliest Event Reported): October 16, 2018**

# RESTORBIO, INC.
**(Exact name of registrant as specified in its charter)**

| **Delaware** | **001-38359** | **81-3305277** |
|---|---|---|
| **(State or other jurisdiction of incorporation)** | **(Commission File Number)** | **(I.R.S. Employer Identification No.)** |

**500 Boylston Street, 12th Floor**
**Boston, MA 02116**
**(Address of principal executive offices, including zip code)**

**(857) 315-5521**
**(Registrant's telephone number, including area code)**

**Not Applicable**
**(Former Name or Former Address, if Changed Since Last Report)**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

**Item 7.01. Regulation FD Disclosure.**

resTORbio, Inc. (the "Company") from time to time presents and/or distributes to the investment community at various industry and other conferences slide presentations to provide updates and summaries of its business. A copy of its current corporate slide presentation (the "Presentation") is attached to this Current Report on Form 8-K as Exhibit 99.1. The Company undertakes no obligation to update, supplement or amend the materials attached hereto as Exhibit 99.1.

The information in Item 7.01 of this Form 8-K, including Exhibit 99.1 attached hereto, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act") or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing.

**Item 8.01 Other Events**

On October 16, 2018, the Company issued a press release titled "resTORbio Announces Additional RTB101 Phase 2b Data Demonstrating Decreased Incidence of Laboratory-Confirmed RTIs with Severe Symptoms, Total Infections and UTIs." A copy of the press release is attached as Exhibit 99.2 to this Form 8-K and incorporated herein by reference.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits

| Exhibit No. | Description |
|---|---|
| 99.1 | Corporate slide presentation of resTORbio, Inc., dated October 16, 2018. |
| 99.2 | Press release issued by resTORbio, Inc. on October 16, 2018. |

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: October 16, 2018

**RESTORBIO, INC.**

By: /s/ Chen Schor
Chen Schor
President and Chief Executive Officer

Exhibit 99.1

# resTORbio™

October 2018

# Forward-looking statements

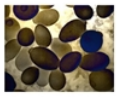
This presentation may contain “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding the safety, efficacy and regulatory and clinical progress of our product candidates, including RTB101 alone and in combination with everolimus. All such forward-looking statements are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. The use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward-looking statements. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, our clinical results and other future conditions. All statements other than statements of historical facts contained in this presentation, including statements regarding future results of operations and financial position, business strategy, current and prospective product candidates, planned clinical trials and preclinical activities, including the initiation, timing, progress and results of our preclinical and clinical studies and our research and development programs, product approvals, research and development costs, current and prospective collaborations, timing and likelihood of success, including our ability to advance RTB101 alone and in combination with everolimus into, and successfully complete, clinical studies, and the timing or likelihood of regulatory filings and approvals, expectations regarding market acceptance and size, plans for launch and commercialization, plans and objectives of management for future operations, and future results of anticipated product candidates, are forward-looking statements. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

These statements are also subject to a number of material risks and uncertainties that are discussed in the section entitled "Risk Factors" in resTORbio’s annual report on Form 10-K for the fiscal year ended December 31, 2017, as well as discussions of potential risks, uncertainties, and other important factors in resTORbio's subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. Neither we, nor our affiliates, advisors, or representatives, undertake any obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

Certain information contained in this presentation relates to or is based on studies, publications, surveys and other data obtained from third-party sources and the Company’s own internal estimates and research. While we believe these third-party sources to be reliable as of the date of this presentation, we have not independently verified, and we make no representation as to the adequacy, fairness, accuracy or completeness of, any information obtained from third-party sources. In addition, all of the market data included in this presentation involves a number of assumptions and limitations, and there can be no guarantee as to the accuracy or reliability of such assumptions. Finally, while we believe our own internal research is reliable, such research has not been verified by any independent source.

# The biology of aging is regulated by TORC1

# TORC1 is an evolutionarily conserved pathway that regulates aging


Yeast


Worms

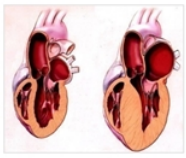

Flies


Mice

TORC1 inhibition extended lifespan and healthspan
and improved the following aging-related conditions in preclinical studies:

Improved
Immune Function

Ameliorate Heart
Failure



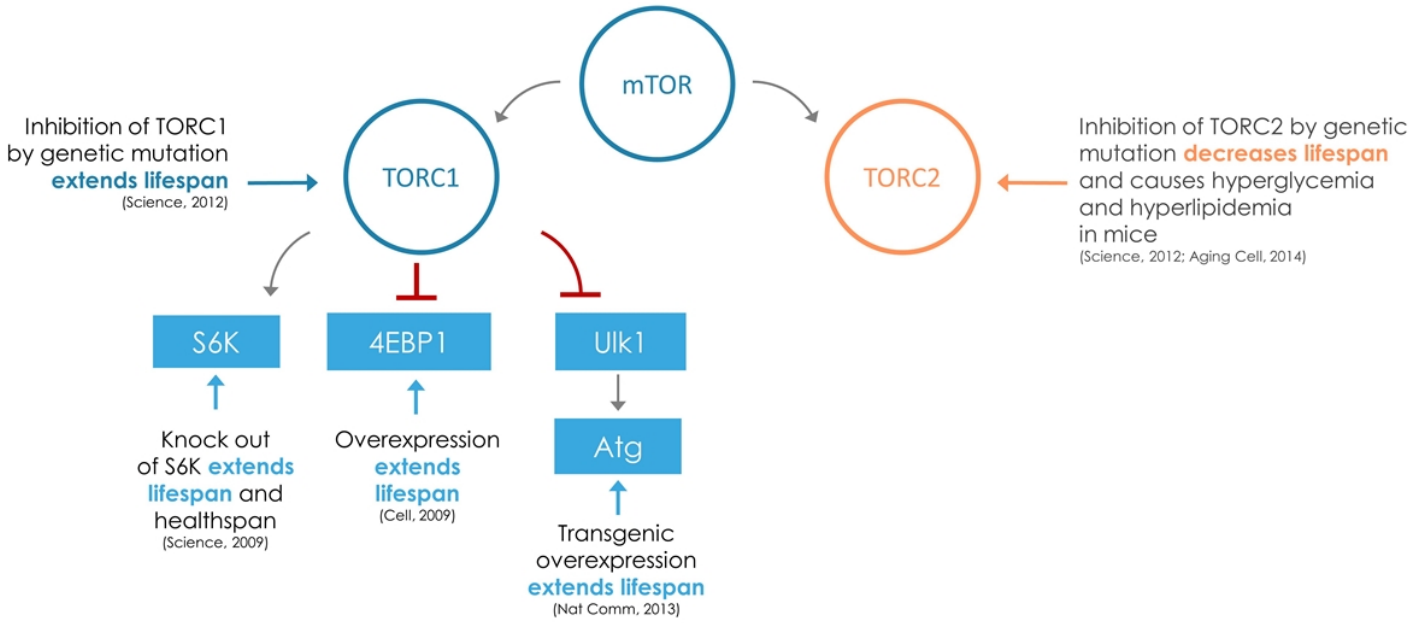
Ameliorate
Neurodegenerative
Diseases

# resTORbio highlights

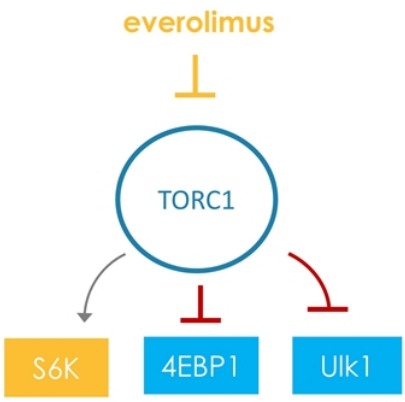
- **Developing first in class and most advanced selective TORC1 program**
  - The lead indication for RTB101, our proprietary TORC1 inhibitor, is to decrease the incidence of respiratory tract infections (RTIs) in high-risk elderly by enhancing the function of the aging immune system
  - RTIs are the 4th leading cause for hospitalization in the 65+; 2nd in 85+ (US)
- **Positive results in Phase 2b study: RTB101 10 mg once daily**
  - 30.6% reduction in the percentage of patients with laboratory-confirmed RTIs (p=0.025)
  - 52.1% reduction in percentage of patients with severe laboratory-confirmed RTI symptoms (p=0.034)
  - 23.6% reduction in the percentage of patients with any infection compared to placebo (p=0.032)
  - Successfully defined dose and patient population for planned pivotal Phase 3 program
- **Data-driven approach to expand into additional indications**
  - Leveraging Phase 2b data to evaluate additional aging-related diseases
  - Initiate Phase 2a trial in additional aging-related disease in Q4 2018/Q1 2019
- **Cash, cash equivalents and marketable securities were ~$125 million as of June 30, 2018**

# TORC1 Pathway

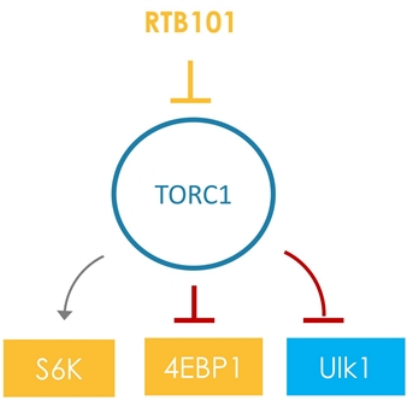
# Selective inhibition of TORC1 may have therapeutic benefit for the treatment of aging-related diseases



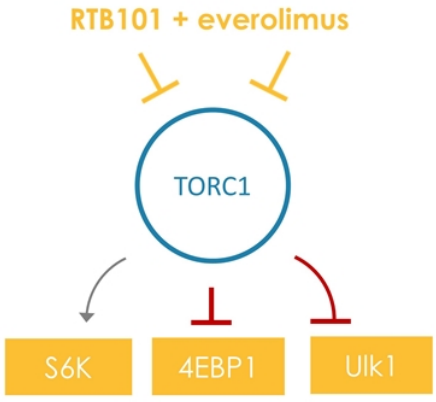
# Spectrum of TORC1 inhibition with everolimus and RTB101




Inhibiting the phosphorylation of 1 target of TORC1




Inhibiting the phosphorylation of 2 targets of TORC1




Inhibiting the phosphorylation of 3 targets of TORC1

= indicates phosphorylation is inhibited

# RTB101 Program

# Results of Phase 2a trial

- 264 mostly healthy elderly people randomized to the following TORC1 inhibitor treatment arms:

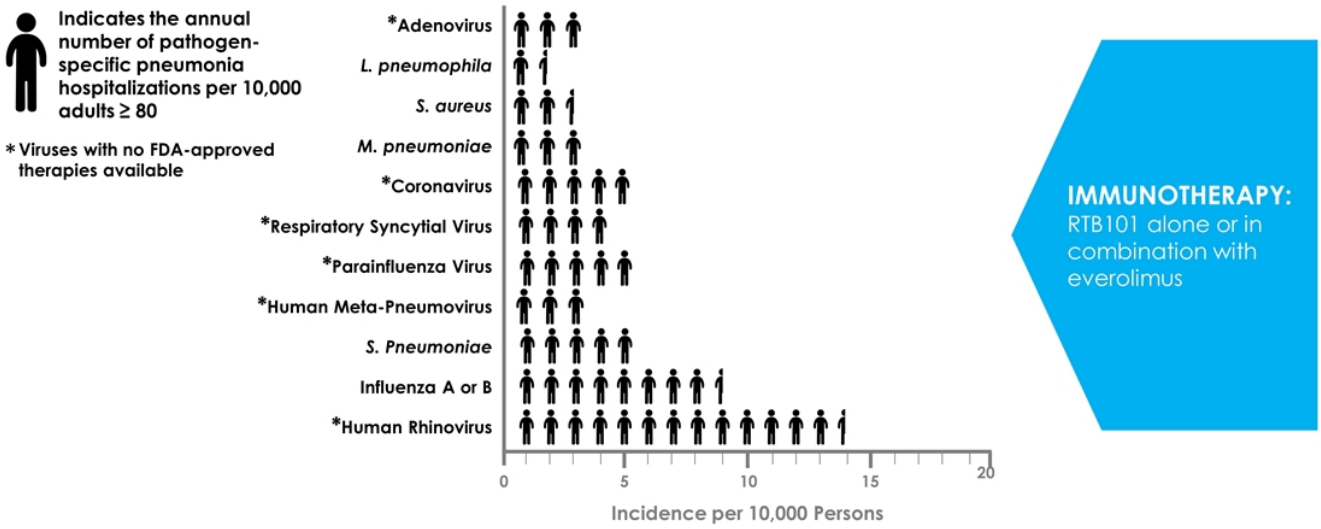

  - Everolimus 0.1 mg + RTB101 10 mg
  - RTB101 10 mg
  - Everolimus 0.5 mg
  - Everolimus 0.1 mg
  - Placebo

- Both RTB101 10 mg QD and RTB101 10 mg + everolimus 0.1 mg QD significantly reduced the incidence of all infections as well as respiratory tract infections (RTIs)
  - Reduction in RTIs:
    - RTB101 10 mg: 42% reduction (p=0.006)
    - RTB101 10 mg + everolimus 0.1 mg: 36% reduction (p=0.01)
- Both RTB101 10 mg and RTB101 10 mg + everolimus 0.1 mg upregulated antiviral gene expression in whole blood

QD = once daily

# RTB101 offers new approach to harnessing the immune system to target multiple pathogens

The majority of pathogens detected in elderly people hospitalized for pneumonia are viruses for which **NO APPROVED THERAPIES** are currently available




**IMMUNOTHERAPY:**
RTB101 alone or in combination with everolimus

Sources: S. Jain et al., NEJM 2015

# Phase 2a to Phase 2b

Phase 2a: 65 and older, 23% with comorbidities

Phase 2b

85 and older

65 and older w/ asthma

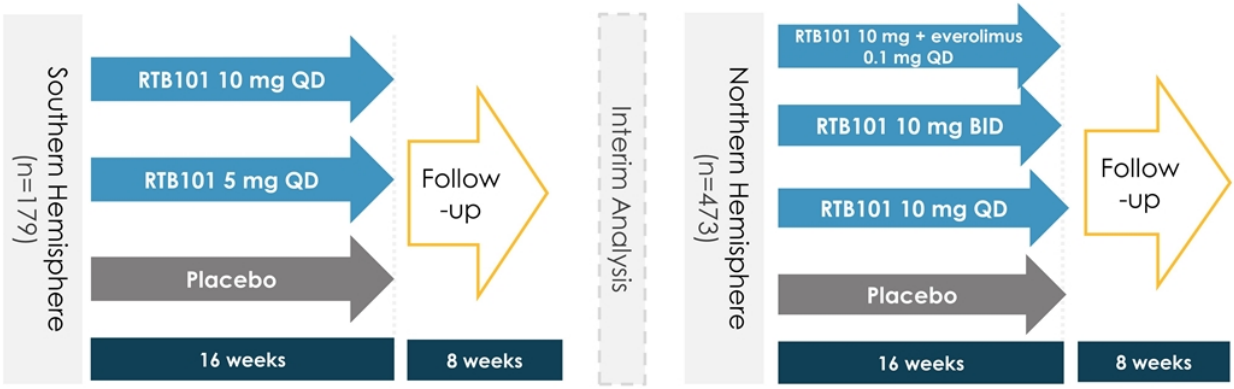
65 and older w/ diabetes

65 and older w/ COPD

65 and older, smokers

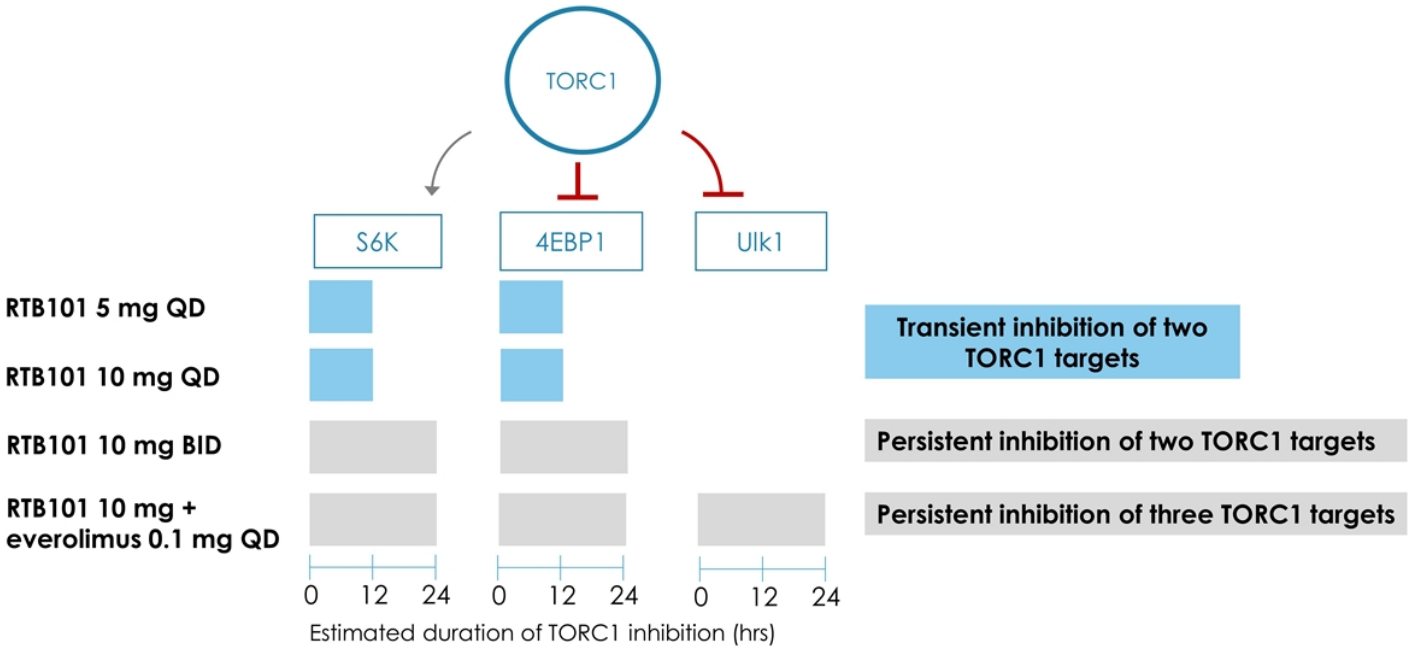
# Phase 2b design

- **Primary Endpoint**: Reduction in the percentage of patients with laboratory-confirmed RTIs through week 16
- **Population**: Elderly subjects at increased risk of RTI-associated morbidity and mortality, defined as:
  - ≥ 85 years of age
  - 65-84 years of age with one or more comorbidities including:
    - Asthma
    - Chronic obstructive pulmonary disease (COPD)
    - Type 2 diabetes mellitus (T2DM)
    - Current smoker



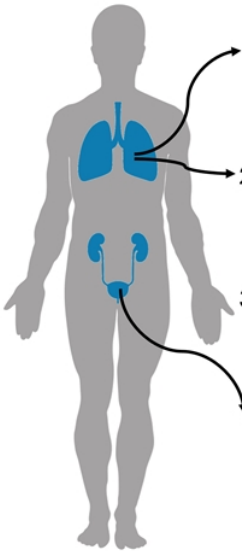

QD = once daily; BID = twice daily

# Dosing regimens in Phase 2b result in different estimated duration and spectrum of TORC1 inhibition




QD = once daily; BID = twice daily

# Phase 2b data supports RTB101 potential efficacy for enhancing immune function and reducing the incidence of infections

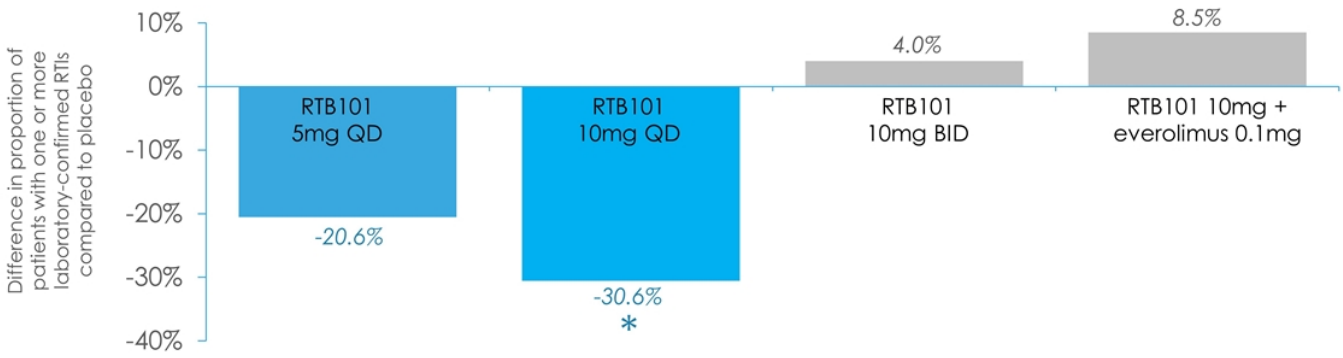


1. **Statistically significant reduction in percent of patients with laboratory-confirmed RTIs (announced Jul 2018)**
   - **30.6%** reduction in the percentage of patients with laboratory-confirmed RTIs in the RTB101 10 mg QD cohort compared to the placebo cohort **(OR=0.601; p=0.025)**
2. **Statistically significant decrease in severity of RTI symptoms (announced Oct 2018)**
   - **52.1%** reduction in the percentage of patients with severe laboratory-confirmed RTI symptoms in the RTB101 10 mg QD cohort compared to the placebo cohort **(OR=0.437; p=0.034)**
3. **Statistically significant decrease in the incidence of total infections of any kind (announced Oct 2018)**
   - **23.6%** reduction in the percentage of patients with any infection (laboratory-confirmed RTIs and all other infections) in the RTB101 10 mg QD cohort compared to placebo cohort **(OR=0.653; p=0.032)**
4. **Statistically significant decrease in the incidence of urinary tract infections (UTIs) (announced Oct 2018)**
   - **74.6%** reduction in the percentage of patients with UTIs in the RTB101 10 mg BID cohort compared to the placebo cohort **(OR=0.211; p=0.027)**
   - **34.4%** reduction in the percentage of patients with UTIs in the RTB101 10 mg QD cohort compared to the placebo cohort **(OR=0.601; p=0.156)**

Statistically significant defined as a nominal p-value < 0.05; QD = once daily; BID = twice daily;
OR = odds ratio which represents the odds of experiencing one or more event in the active treatment group versus the placebo group;

# RTI Data

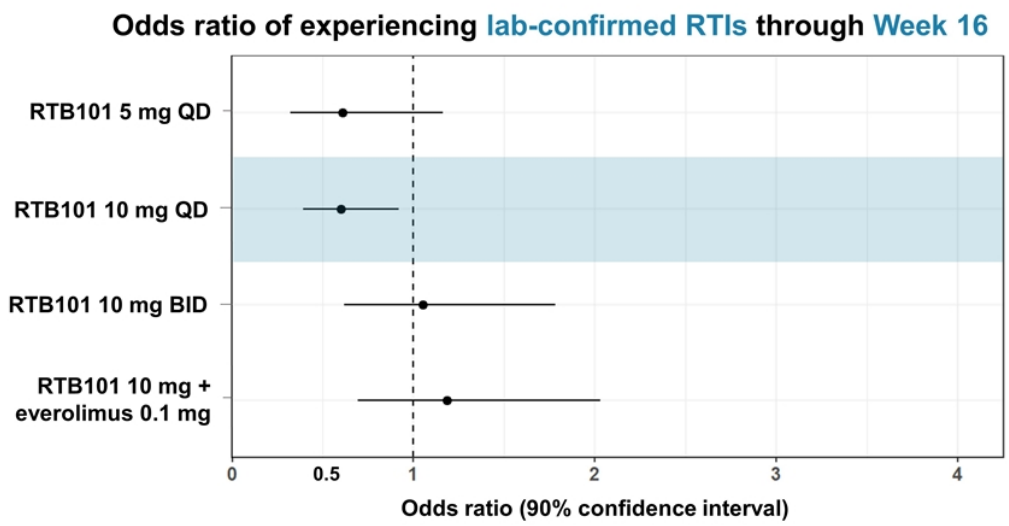
# A significant reduction in the percentage of patients with laboratory-confirmed RTIs was observed in the RTB101 10 mg QD cohort




| | | RTB101 5mg QD | RTB101 10mg QD | RTB101 10mg BID | RTB101 10mg + everolimus 0.1mg |
|---|---|---|---|---|---|
| **p-value[1]** | | **0.109** | **0.025** | **0.560** | **0.700** |
| **Odds ratio[2] (CI[3])** | | **0.618** (0.325; 1.176) | **0.601** (0.391; 0.922) | **1.050** (0.618; 1.782) | **1.187** (0.694; 2.030) |
| **Active** | **N** | 61 | 176 | 120 | 115 |
| | **$N_{RTI}$[4]** | 21 | 34 | 25 | 25 |
| **Placebo** | **N** | 60 | 180 | 120 | 120 |
| | **$N_{RTI}$[4]** | 26 | 50 | 24 | 24 |

[1]One-sided p-value; [2]Odds ratio represents the odds of experiencing one or more laboratory-confirmed RTIs in the active treatment group versus the placebo group; [3]90% confidence interval; [4]No. of patients in cohort with one or more laboratory-confirmed RTIs; *p<0.05; QD = once daily; BID = twice daily

# Odds ratio further supports dose selection and potential efficacy of RTB101 10 mg QD

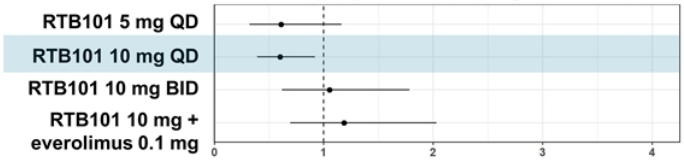



| | Active rate[1] | Placebo rate[1] | Odds ratio[2] | p-value[3] |
|---|---|---|---|---|
| RTB101 5 mg QD | 21/61 | 26/60 | 0.618 | 0.109 |
| RTB101 10 mg QD | 34/176 | 50/180 | 0.601 | 0.025* |
| RTB101 10 mg BID | 25/120 | 24/120 | 1.050 | 0.560 |
| RTB101 10 mg + everolimus 0.1 mg | 25/115 | 24/120 | 1.187 | 0.700 |

[1]No. of patients in cohort with one or more laboratory-confirmed RTIs/No. of patients in cohort; [2]Odds ratio represents the odds of experiencing one or more laboratory-confirmed RTIs in the active treatment group versus the placebo group; [3]One-sided p-value; *p<0.05; QD = once daily; BID = twice daily

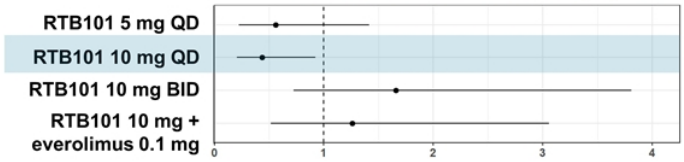
# RTB101 10 mg QD showed consistent benefit in multiple pre-specified analyses of lab-confirmed RTIs

**Odds ratio of experiencing lab-confirmed RTIs through Week 16 – primary endpoint**



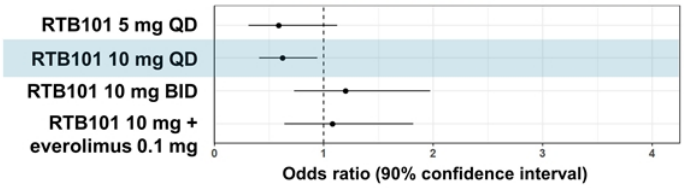

**OR =0.601; p=0.025**

**Odds ratio of experiencing severe lab-confirmed RTI symptoms through Week 16**




**OR =0.437; p=0.034**

**Odds ratio of experiencing lab-confirmed RTIs through Week 24**

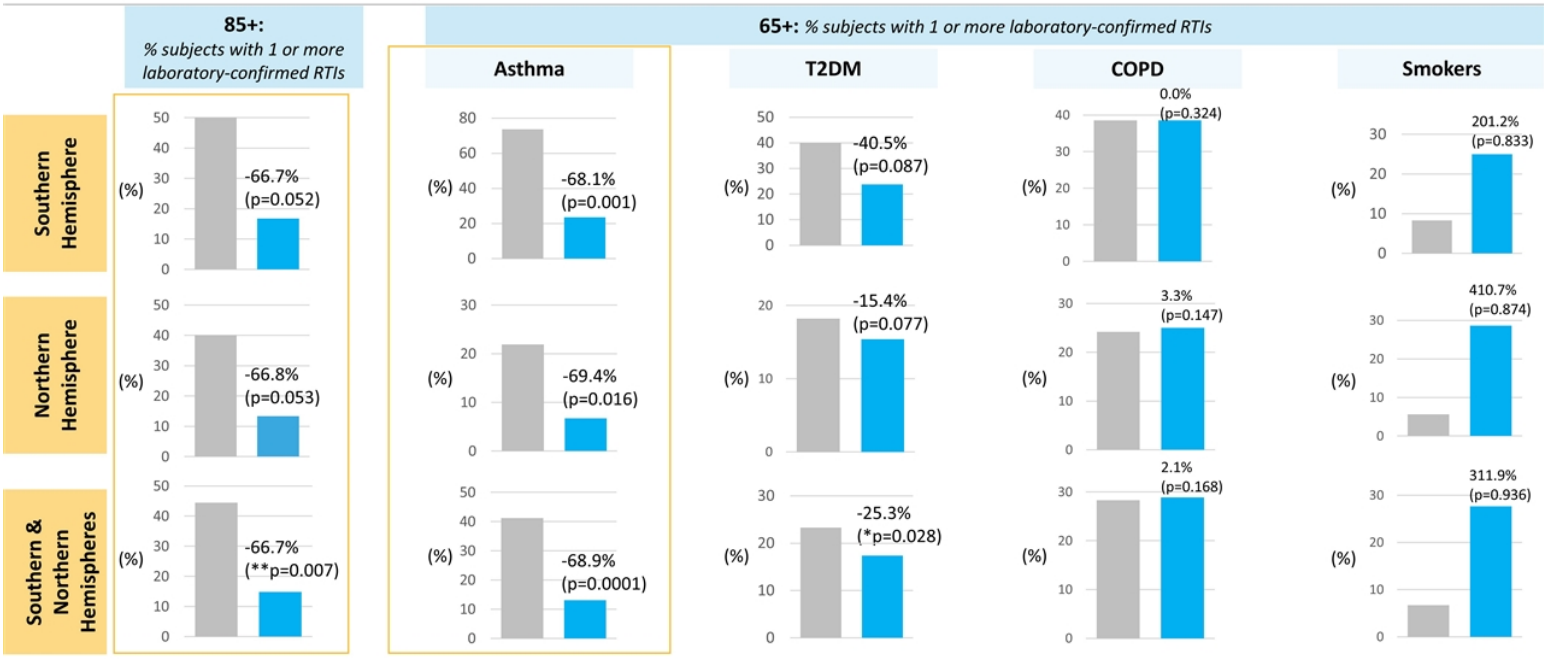



**OR =0.623; p=0.030**

RTB101 10 mg QD associated with statistically significant reductions across three different analyses of laboratory-confirmed RTIs: Week 16, severe RTIs and Week 24

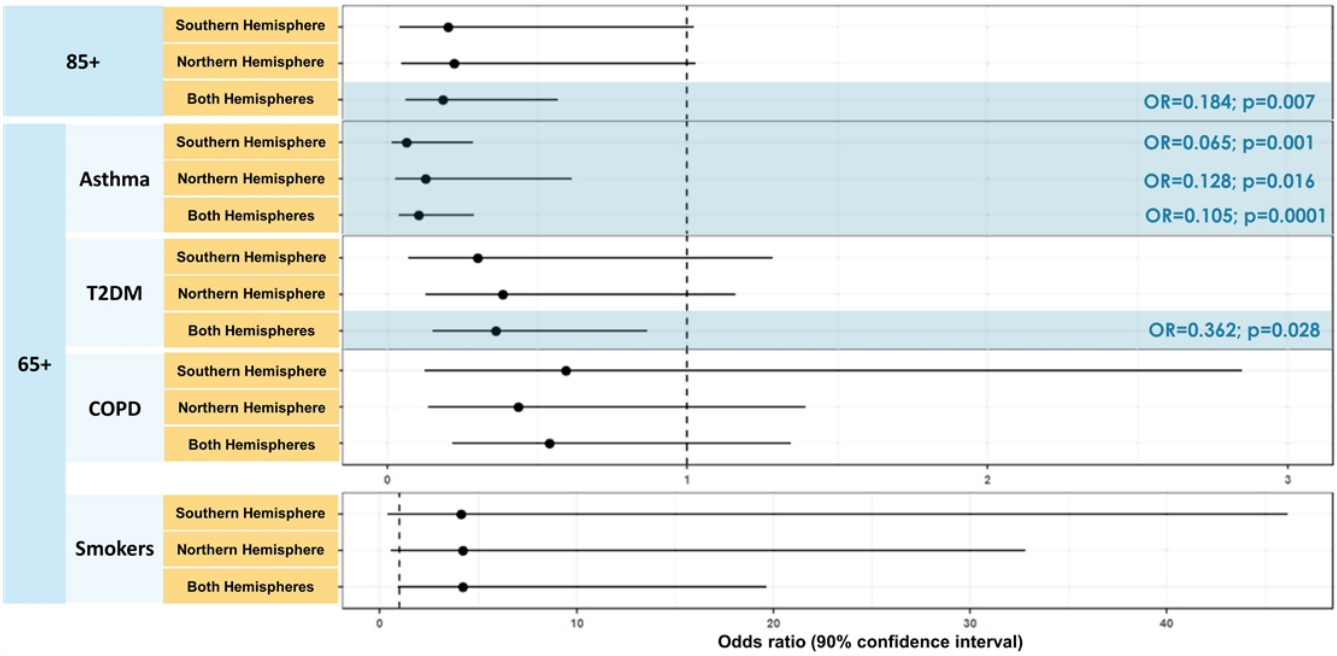
One-sided p-value; QD = once daily; BID = twice daily;
Odds ratio represents the odds of experiencing one or more event in the active treatment group versus the placebo group

# RTB101 10 mg QD showed the greatest effect in the 85+ and 65+ with asthma subpopulations (pre-specified analyses)




= placebo cohort; = RTB101 10mg once daily cohort; One-sided p-value; QD = once daily; BID = twice daily

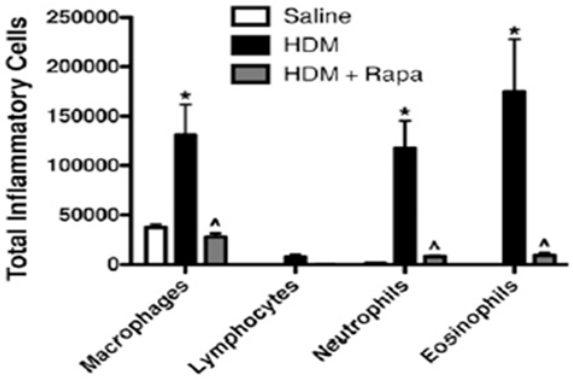
# Odds ratio further supports effect seen in the 85+ and 65+ with asthma subpopulations




OR = Odds ratio represents the odds of experiencing one or more laboratory-confirmed RTIs in the active treatment group versus the placebo group; One-sided p-value

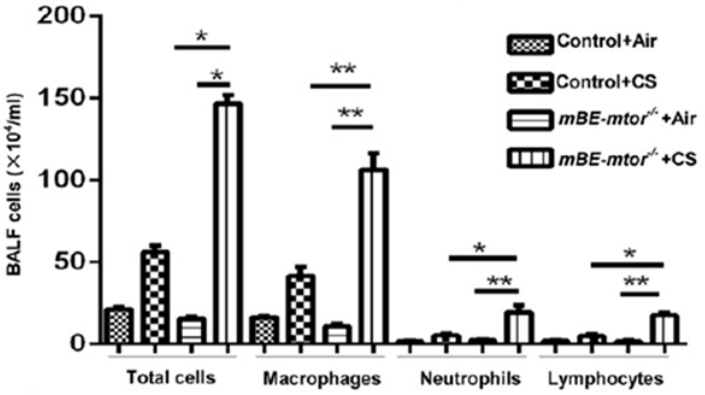
# Preclinical data: mTOR inhibition decreased airway inflammation in asthma and increased airway inflammation due to smoking

**Asthma**




mTOR inhibition with rapamycin (Rapa) significantly **decreased** airway inflammation in a preclinical asthma model in which mice were exposed to intranasal house dust mites (HDM)[1]

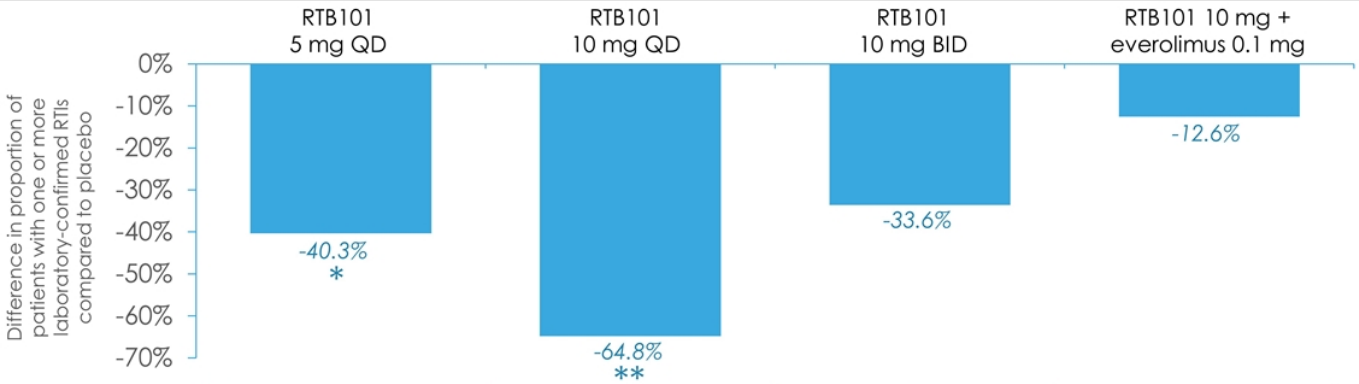
**Smoking**




Disruption of mTOR selectively in bronchial epithelial cells (mBE-mtor$^{-/-}$) significantly **increased** cigarette smoke (CS)-induced lung inflammation in a COPD model in which mice were exposed to cigarette smoke for 6 months[2]

[1]Mushaben E. M. *et al., J Immunol* 2011:187:5756-5763; [2] Wang Y *et al., J Immunol* 2018;200:2571-2580; *p<0.05, **p<0.01

# High responder population identified as non-smoking subjects who are 85+ and 65+ with asthma

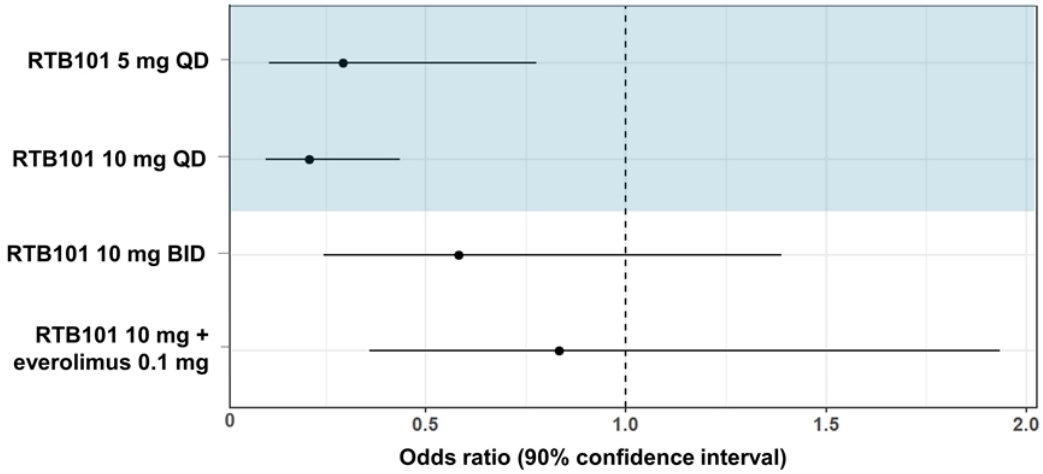



| | | | | | |
|---|---|---|---|---|---|
| **p-value[1]** | | **0.019** | **0.0002** | **0.153** | **0.361** |
| **Odds ratio[2] (CI[3])** | | **0.292** (0.109; 0.777) | **0.208** (0.099; 0.435) | **0.582** (0.244; 1.389) | **0.833** (0.359; 1.934) |
| **Active** | **N** | 30 | 69 | 42 | 40 |
| | **$N_{RTI}$[4]** | 11 | 10 | 8 | 10 |
| **Placebo** | **N** | 26 | 68 | 42 | 42 |
| | **$N_{RTI}$[4]** | 16 | 28 | 12 | 12 |

[1]One-sided p-value; [2]Odds ratio represents the odds of experiencing one or more laboratory-confirmed RTIs in the active treatment group versus the placebo group; [3]90% confidence interval; [4]No. of patients in cohort with one or more laboratory-confirmed RTIs; *p<0.05, **p<0.001; QD = once daily; BID = twice daily

# Odds ratio further supports effect of RTB101 10 mg QD in non-smoking subjects who are 85+ and 65+ with asthma

**Odds ratio of experiencing lab-confirmed RTIs through Week 16 in non-smoking subjects 85+ and 65+ with asthma**

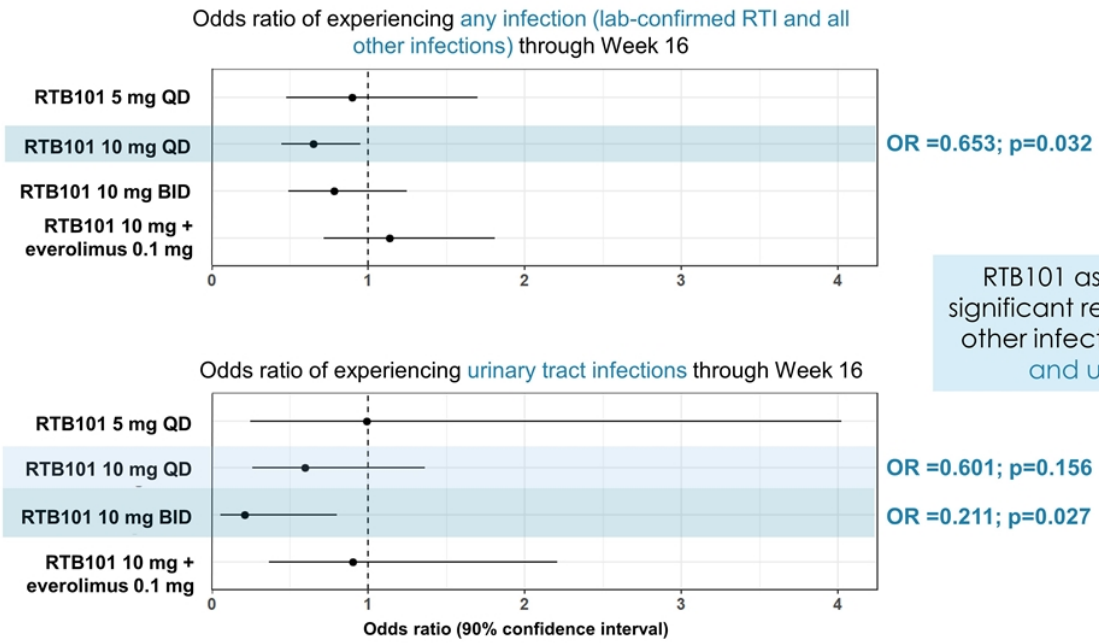



| Active rate[1] | Placebo rate[1] | Odds ratio[2] | p-value[3] |
|---|---|---|---|
| 11/30 | 16/26 | 0.292 | 0.019* |
| 10/69 | 28/68 | 0.208 | 0.0002** |
| 8/42 | 12/42 | 0.582 | 0.153 |
| 10/40 | 12/42 | 0.833 | 0.361 |

[1]No. of patients in cohort with one or more laboratory-confirmed RTIs/No. of patients in cohort; [2]Odds ratio represents the odds of experiencing one or more laboratory-confirmed RTIs in the active treatment group versus the placebo group; [3]One-sided p-value; *p<0.05, **p<0.001; QD = once daily; BID = twice daily

# Other Infection Data

# RTB101 may also reduce the incidence of total infections of any kind







RTB101 associated with statistically significant reductions across analyses of other infections: Infections of any kind and urinary tract infections

OR = Odds ratio represents the odds of experiencing one or more infections in the active treatment group versus the placebo group; One-sided p-value; QD = once daily; BID = twice daily

# RTB101 was well-tolerated in high-risk elderly patients through Week 24

- Adverse events (AEs) were balanced between the RTB101 10 mg QD and placebo cohorts
- 1 unrelated death occurred in the RTB101 10 mg QD cohort (patient was hit by car while riding a bicycle), 1 unrelated death occurred in the RTB101 10 mg BID cohort and 1 unrelated death occurred in the placebo cohort (both from unknown causes)

| | RTB101 10 mg QD | Placebo |
|---|---|---|
| **Serious AEs** (% of patients) | 4.5% | 7.8% |
| **Discontinued study drug due to an AE** (% of patients) | 5.1% | 5.6% |
| **Number of severe AEs** | 12 | 25 |

QD = once daily; BID = twice daily

# Summary of Phase 2b RTI data

- **The study successfully identified the dose and patient population for pivotal trials**:
  - Dose: **RTB101 10 mg QD**:
    - 30.6% reduction in the percentage of patients with laboratory-confirmed RTIs (p=0.025)
    - 52.1% reduction in percentage of patients with severe laboratory-confirmed RTI symptoms (p=0.034)
    - 23.6% reduction in the percentage of patients with any infection compared to placebo (p=0.032)
  - Patient population:
    - **65 and older with asthma:** 68.9% reduction in laboratory-confirmed RTIs (p=0.0001)
    - **85 and older:** 66.7% reduction in laboratory-confirmed RTIs (p=0.007)
- All doses, including RTB101 10 mg QD, were well-tolerated
- **End-of-Phase 2 meeting expected in Q4 2018/Q1 2019**
- **Plan to initiate pivotal trials in 1H 2019**

QD = once daily; BID = twice daily

# Medical Need & Market Opportunity

# RTIs represent a significant healthcare burden

- RTIs are the 4th most common cause for hospitalization in 65+ (2nd in 85+)[1]
- RTIs are the 7th leading cause of death in 65+ (5th in 85+)[2]
- Mortality from RTIs is higher than mortality from colorectal, pancreatic, breast or prostate cancer[3]
- RTIs cause the majority of asthma exacerbations in the elderly[4]
- The majority of RTIs are caused by viruses for which there are no approved therapies[5]
- Decreasing the incidence of RTIs in the elderly may significantly decrease health care costs

[1]Pfunter, A (2013) HCUP Statistical Brief #162; [2]Kochanek KD, Murphy SL, Xu JQ, Tejada-Vera B. Deaths: Final data for 2014. National vital statistics reports; vol 65 no 4. Hyattsville, MD: National Center for Health Statistics. 2016; [3] 2017, NCHS. National Vital Statistics System. Mortality; 2015. American Cancer Society, Inc. Surveillance Research; [4] BMJ. 1995. 310:1225-1228; BMJ. 1993. 307:982-986; [5] Am J Med. 2002. 112(6A):42S-49S;

# Estimated number of people 85+ and 65+ with asthma in key geographies

| | US | EU5 | JP | CN |
|---|---|---|---|---|
| **Elderly (65-84 years old) with asthma:** | 3.2M | 3.3M[1] | 2.1M[2] | 2.5M[3] |
| **Very elderly (85+ years old):** | 6.5M | 9.3M | 5.5M | 8.9M |
| **# Elderly People** | **10M** | **13M** | **8M** | **11M** |

Market size across key geographies estimated at 42M

[1]Based on estimated percentage of asthmatics in older adults in high-income countries. [2]Based on percentage of asthmatics in the Japanese adult population. [3]Based on percentage of adults age ≥60 on asthma medication in Jinan province; Likely underestimated due to low diagnosis rate of asthma

# Survey of 100 physicians to determine potential usage in the target patient populations

**Physician survey*: Expected use in target populations**

| % Reduction in RTI | Estimated % prescribed in patients (patient-weighted means) | | |
|---|---|---|---|
| | ≥85 | 65-84 with asthma | 65-84 with comorbidities |
| **25%** | 33% | 36% | 36% |
| **33%** | 41% | 44% | 47% |
| **40%** | **46%** | **48%** | **51%** |

***Respondent background (n=100):**

| Medical Specialty | |
|---|---|
| Geriatrics | 25 |
| Primary Care | 50 |
| Pulmonologist | 25 |

| Practice characteristics | |
|---|---|
| Years practicing medicine | Avg 19 *(median 19.5, range 6-33)* |
| # pts ≥ 65 seen/month | Avg 250 *(median 220, range 80-600)* |
| % services billed to Medicare | Avg 63% *(median 65%, range 30-100%)* |

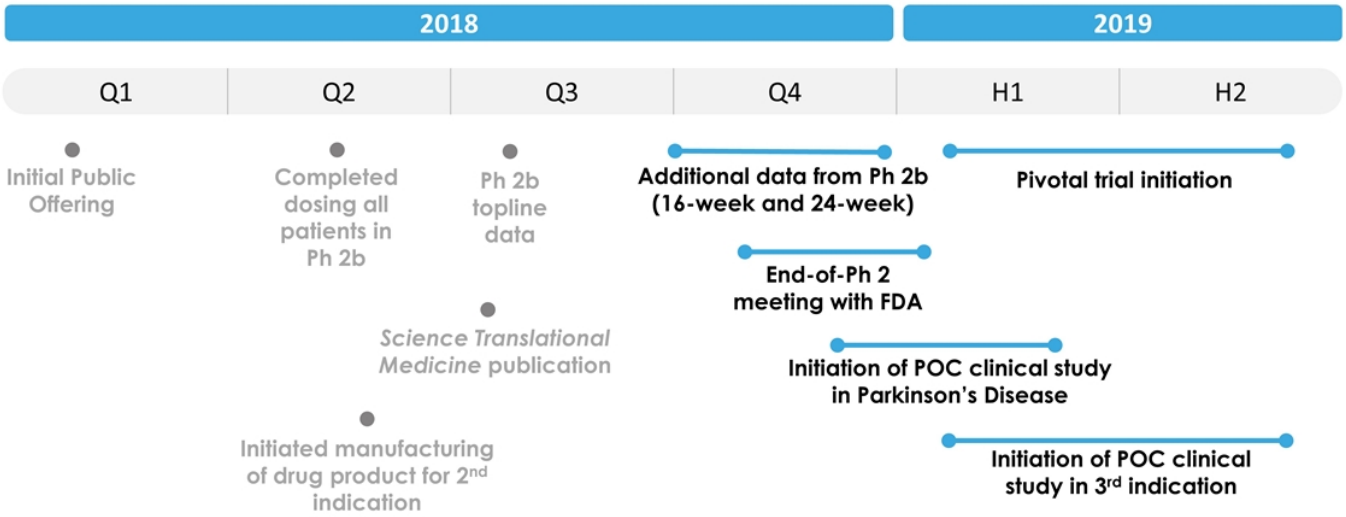
# Most advanced pipeline targeting aging-related diseases

| Program | Indication | Discovery | Preclinical | Phase 1 | Phase 2 | Phase 3 | Anticipated Milestones |
|---|---|---|---|---|---|---|---|
| **RTB101** | **Respiratory Tract Infections** | | | | | | **End-of-Phase 2 meeting planned for 4Q18/1Q19** |
| **RTB101+ rapalog** | **Parkinson's Disease** | | | | | | **Initiate Phase 2a 4Q18/1Q19**** |
| RTB101 or RTB101 + rapalog | Other Infections* | | | | | | **Signal detection for these indications in the Phase 2b 4Q18**** |
| | Heart Failure with Preserved Ejection Fraction | | | | | | |
| Undisclosed TORC1 inhibitor | Undisclosed | | | | | | |

* *Other infections include those that the elderly are at increased risk of contracting, such as urinary tract infections.*

** *For heart failure with preserved ejection fraction, Parkinson's Disease and certain other infections, we may be required to file an investigational new drug application, or IND, prior to initiating Phase 2 clinical trials. We expect to have the ability to initiate these Phase 2 clinical trials without the need to conduct prior Phase 1 trials.*

# Near term planned clinical milestones and path forward




POC = proof of concept

# resTORbio highlights

- **Developing first in class and most advanced selective TORC1 program**
  - The lead indication for RTB101, our proprietary TORC1 inhibitor, is to decrease the incidence of RTIs in high-risk elderly by enhancing the function of the aging immune system
  - RTIs are the 4th leading cause for hospitalization in the 65+; 2nd in 85+ (US)
- **Positive results in Phase 2b study: RTB101 10 mg once daily**
  - 30.6% reduction in the percentage of patients with laboratory-confirmed RTIs (p=0.025)
  - 52.1% reduction in percentage of patients with severe laboratory-confirmed RTI symptoms (p=0.034)
  - 23.6% reduction in the percentage of patients with any infection compared to placebo (p=0.032)
  - Successfully defined dose and patient population for planned pivotal Phase 3 program
- **Data-driven approach to expand into additional indications**
  - Leveraging Phase 2b data to evaluate additional aging-related diseases
  - Initiate Phase 2a trial in additional aging-related disease in Q4 2018/Q1 2019
- **Cash, cash equivalents and marketable securities were ~$125 million as of June 30, 2018**

resTORbio™

October 2018

**resTORbio Announces Additional RTB101 Phase 2b Data Demonstrating Decreased Incidence of Laboratory-Confirmed RTIs with Severe Symptoms, Total Infections and UTIs**

*- RTB101 10 mg once daily decreased incidence of laboratory-confirmed respiratory tract infections (RTIs) with severe symptoms by 52.1% vs. placebo (p=0.034) -*

*- In addition to decreasing the incidence of laboratory-confirmed RTIs by 30.6% vs. placebo (p=0.025), RTB101 10 mg once daily decreased incidence of total infections of any kind by 23.6% vs. placebo (p=0.032) -*

*- RTB101 10 mg twice daily and once daily decreased incidence of urinary tract infections (UTIs) by 74.6% (p=0.027) and 34.4% (p=0.156), respectively, vs. placebo -*

*- Company expects to initiate pivotal Phase 3 program with RTB101 10 mg once daily in RTIs in 1H19 -*

**BOSTON, October 16, 2018 —** resTORbio, Inc. **(Nasdaq: TORC)** today announced additional positive results from its dose-ranging Phase 2b clinical trial of RTB101, an oral, selective and potent inhibitor of target of rapamycin complex 1 (TORC1), in respiratory tract infections (RTIs), as well as results from pre-specified analyses for any infection and urinary tract infections (UTIs). Analysis of secondary and exploratory endpoints expands upon positive topline results announced in July 2018, which demonstrated a statistically significant 30.6% reduction in the percentage of patients with laboratory-confirmed RTIs during the 16-week treatment period in the RTB101 10 mg once daily dosing cohort compared to the placebo cohort (p=0.025).

“RTB101 10 mg once daily was observed to decrease not only the incidence of RTIs, but also the severity of RTI symptoms. We look forward to advancing this dose in a planned Phase 3 program for reducing the incidence of RTIs in high-risk elderly patients,” said Joan Mannick, M.D., Co-Founder and Chief Medical Officer of resTORbio. “These data also demonstrate that RTB101 may decrease the incidence of other infections in the elderly, including UTIs. It remains to be determined if the most efficacious RTB101 dose for preventing RTIs and UTIs differs because UTIs occur in a different organ system and are caused by different pathogens than RTIs. We continue to develop our clinical strategy for UTIs, including dose selection.”

resTORbio reported the following additional pre-specified analyses of secondary and exploratory endpoints:

**Severity and incidence of laboratory-confirmed RTIs:**

- **Decreased severity of laboratory-confirmed RTI symptoms during 16 weeks of study drug treatment** (severe RTI symptoms were defined as those that prevent normal activities):
  - **RTB101 10 mg once daily**: 52.1% reduction in the percentage of patients with severe laboratory-confirmed RTI symptoms compared to placebo (odds ratio [OR]=0.437; p=0.034).
  - **RTB101 5 mg once daily**: 41.3% reduction in the percentage of patients with severe laboratory-confirmed RTI symptoms compared to placebo (OR=0.560; p=0.152).
  - No reduction in the percentage of patients with severe laboratory-confirmed RTI symptoms was observed with RTB101 10 mg twice daily or with RTB101 in combination with everolimus.
- **Decreased incidence of laboratory-confirmed RTIs during 16 weeks of study drug treatment and an additional 8 weeks of follow-up off study drug (for a total of 24 weeks**):
  - **RTB101 10 mg once daily:** 27.5% reduction in the percentage of patients with laboratory-confirmed RTIs compared to placebo during the 24 weeks (OR=0.623; p=0.030).

- **RTB101 5 mg once daily:** 19.3% reduction in the percentage of patients with laboratory-confirmed RTIs compared to placebo during the 24 weeks (OR=0.604; p=0.097).
- No reduction in the percentage of patients with laboratory-confirmed RTIs was observed with RTB101 10 mg twice daily or with RTB101 in combination with everolimus.

**Incidence of total infections of any kind (laboratory-confirmed RTIs and other infections) during 16 weeks of study drug treatment:**

- **Decreased incidence of any infections:**
  - **RTB101 10 mg once daily***:* 23.6% reduction in the percentage of patients with any infection compared to placebo (OR=0.653; p=0.032).
  - **RTB101 10 mg twice daily:** 15.4% reduction in the percentage of patients with any infection compared to placebo (OR=0.780; p=0.192).
  - No reduction in the percentage of patients with any infection was observed with RTB101 5 mg once daily or with RTB101 in combination with everolimus.

**Incidence of UTIs during 16 weeks of study drug treatment:**

- **Decreased incidence of UTIs:**
  - **RTB101 10 mg once daily:** 34.4% reduction in the percentage of patients with one or more UTIs compared to placebo (OR=0.601; p=0.156).
  - **RTB101 10 mg twice daily:** 74.6% reduction in the percentage of patients with one or more UTIs compared to placebo (OR= 0.211; p=0.027).
  - No reduction in the percentage of patients with one or more UTIs was observed with RTB101 5 mg once daily or with RTB101 in combination with everolimus.

Consistent with prior Phase 2b data, RTB101 was observed to be safe and well-tolerated.

Detailed results from this Phase 2b trial will be submitted for presentation at an upcoming medical meeting.

“These data further support the potential of RTB101 as a novel therapy to enhance immune function and reduce the incidence of RTIs in the elderly, which are a leading cause of hospitalization and mortality in the elderly. The activity of RTB101 in each patient subgroup in the study was consistent with what was observed in previously reported data from this trial,” said Chen Schor, Co-Founder, President and CEO of resTORbio. “We look forward to our end-of-Phase 2 meeting with the U.S. Food and Drug Administration and expect to initiate our pivotal Phase 3 program to reduce the incidence of RTIs in the first half of 2019.”

A corporate presentation, which includes resTORbio’s Phase 2b trial results, can be found on the Company’s website at: http://ir.restorbio.com/news-and-events/presentations.

**Phase 2b Trial Design**

The purpose of the exploratory dose-finding, randomized, double-blind, placebo-controlled, multi-center Phase 2b clinical trial was to determine if RTB101 alone or in combination with everolimus decreased the incidence of RTIs in high-risk elderly patients, as well as to evaluate safety and tolerability alone or in combination with everolimus, to support dose selection for pivotal trials.

The study enrolled 652 patients at increased risk of morbidity and mortality from RTIs including patients who were: (i) 85 years of age or older, or (ii) 65 years of age or older with asthma, type 2 diabetes (T2DM), chronic obstructive pulmonary disease (COPD) or current smokers. The study consisted of two parts. Part 1 was

conducted during the winter cold and flu season in the southern hemisphere and 179 elderly patients were randomized to receive either placebo, RTB101 5 mg once daily or RTB101 10 mg once daily. At the end of Part 1, an interim analysis was conducted by an unblinded data monitoring committee who selected the RTB101 10 mg once daily dose to move forward into Part 2 of the study. Part 2 was conducted during the winter cold and flu season in the northern hemisphere and 473 elderly patients were randomized to receive either placebo, RTB101 10 mg once daily, RTB101 10 mg twice daily, or RTB101 10 mg in combination with everolimus 0.1 mg once daily. All patients were treated with study drug for 16 weeks, and then were followed for an additional 8 weeks off study drug.

The primary endpoint of the trial was a reduction, as compared to placebo, in the percentage of patients with one or more laboratory-confirmed RTIs during the 16 weeks of study drug treatment. A pre-specified exploratory endpoint was a reduction, as compared to placebo, in the percentage of patients with one or more laboratory-confirmed RTIs in each of the patient subgroups (³ 85 years of age, ³ 65 years of age with asthma, COPD, T2DM, or current smokers). Statistical significance was defined as a nominal p-value below 0.05. Per the protocol, a pre-specified analysis included all patients enrolled in both Part 1 and Part 2 to increase the power of the study.

Additional information about the study [NCT03373903] can be obtained at www.ClinicalTrials.gov.

**About Respiratory Tract Infections**

The reduced ability of the aging immune system to effectively detect and fight infections results in increased susceptibility of the elderly to RTIs. In the U.S., RTIs are the fourth leading cause of hospitalizations and seventh leading cause of death in people age 65 years and older. Additionally, the majority of asthma exacerbations are caused by RTIs, and the majority of RTIs are caused by viruses for which there are no currently approved therapies.

**About Urinary Tract Infections**

UTIs are one of the most common bacterial infections in the elderly, especially in women. Nearly 10% of women age 65 and older and nearly 30% of women age 85 and over report having an UTI annually. In 2011, UTI-related hospitalizations in the U.S. resulted in a total of $2.8 billion in healthcare costs. Currently, continuous daily low-dose antibiotic prophylactic regimens are used to prevent recurrence of symptomatic UTIs and contribute to the development of multidrug resistant bacteria.

**About RTB101**

RTB101 is an oral, selective, and potent inhibitor of TORC1. RTB101 inhibits the phosphorylation of multiple targets downstream of TORC1. Inhibition of TORC1 has been observed to extend lifespan and healthspan in aging preclinical species and to enhance immune, cardiac and neurologic functions, suggesting potential benefits in several aging-related diseases.

**About resTORbio**

resTORbio, Inc. is a clinical stage biopharmaceutical company targeting TORC1 and other biological pathways that regulate aging to develop innovative medicines with the potential to extend healthy lifespan. resTORbio's lead program is selectively targeting TORC1, an evolutionarily conserved pathway that contributes to the decline in function of multiple organ systems, including the immune, cardiovascular and central nervous systems.

**Forward Looking Statements**

This press release contains forward-looking statements that are made pursuant to the safe harbor provisions of the federal securities laws. Investors are cautioned that statements in this press release which are not strictly historical statements, including, without limitation, express or implied statements or guidance regarding our plans to develop and commercialize RTB101 alone or in combination with everolimus, including the therapeutic potential and clinical benefits thereof and the potential patient populations that may be addressed by our product candidates, our ongoing and future clinical trials for RTB101 alone or in combination with everolimus, including the timing of the initiation and anticipated results of these trials, the intended regulatory path for our product candidates and interactions with regulatory authorities, our ability to replicate results achieved in our clinical trials in any future trials, as well as our ability to conduct an end-of-Phase 2 meeting with the U.S. Food and Drug Administration,constitute forward-looking statements identified by words like “believe,” “expect,” “may,” “will,” “should,” “seek,” “anticipate,” or “could” and similar expressions. Such forward-looking statements are subject to a number of risks and uncertainties that could cause actual results to differ materially from those anticipated, including, without limitation, risks associated with: the delay of any planned clinical trials and/or development of RTB101, either alone or in combination with everolimus; our ability to successfully demonstrate the efficacy and safety of our lead product candidate; the clinical results for our lead product candidate which may not support further development of additional indications; uncertainties related to the results of our clinical trials not being predictive of future results in connection with future trials; and obtaining, maintaining and protecting our intellectual property; as well as those risks more fully discussed in the section entitled “Risk Factors” in the Annual Report on Form 10-K filed by resTORbio, Inc. with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent our views only as of today and should not be relied upon as representing its views as of any subsequent date. resTORbio explicitly disclaims any obligation to update any forward-looking statements.

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