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

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FORM 8-K

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

Date of Report (Date of earliest event reported): July 25, 2018

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**RESTORBIO, INC.**

(Exact name of registrant as specified in its charter)

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Delaware  
(State or other jurisdiction  
of incorporation)

001-38359  
(Commission  
File Number)

81-3305277  
(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)

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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.

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**Item 7.01. Regulation FD Disclosure.**

On July 25, 2018, resTORbio, Inc. (the "Company") issued a press release titled, "resTORbio Announces Positive Topline Results in Phase 2b Trial of RTB101" (the "Press Release"). A copy of the Press Release is attached hereto as Exhibit 99.1. In the Press Release, the Company, among other things, announced that it will hold a conference call to discuss the results of the trial. A copy of the corporate slide presentation related to that conference call is attached hereto as Exhibit 99.2.

The information in Item 7.01 of this Form 8-K, including Exhibits 99.1 and 99.2 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 9.01. Financial Statements and Exhibits.**

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Press Release, issued by the Registrant on July 25, 2018.</a>
99.2	<a href="#">Corporate slide presentation dated July 25, 2018.</a>

\* \* \*

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.

resTORbio, Inc.

Date: July 25, 2018

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

**resTORbio Announces Positive Topline Results in Phase 2b Trial of RTB101**

— Statistically significant and clinically meaningful 30.6% reduction in the percentage of patients with one or more laboratory-confirmed respiratory tract infections (RTIs), the primary endpoint of the trial, in the RTB101 10 mg once daily cohort compared to the placebo cohort —

— Statistically significant 68.4% reduction in the incidence of laboratory-confirmed RTIs in the pre-specified analysis of asthma patients 65 years and older treated with RTB101 10 mg once daily —

— Statistically significant 66.7% reduction in the incidence of laboratory-confirmed RTIs in the pre-specified analysis of patients 85 years and older treated with RTB101 10 mg once daily —

— All doses were well-tolerated; RTB101 10 mg once daily had a comparable safety profile to placebo —

— The Phase 2b trial successfully identified a dose and patient populations with high unmet need for upcoming pivotal trials —

— Conference call 8:30 AM Eastern Time today —

**BOSTON, July 25, 2018** — [resTORbio, Inc.](#) (Nasdaq: TORC) today announced positive topline results from its dose-ranging Phase 2b clinical trial that enrolled 652 elderly patients at increased risk of morbidity and mortality associated with respiratory tract infections (RTIs). In this trial, RTB101, an oral, selective, and potent inhibitor of target of rapamycin complex 1 (TORC1), demonstrated a statistically significant and clinically meaningful reduction in the percentage of patients with one or more laboratory-confirmed RTIs during the 16-week treatment period compared to placebo, the primary endpoint of the study, with the 10 mg once daily dose. Greater TORC1 inhibition with RTB101 10 mg in combination with everolimus 0.1 mg did not meet the primary endpoint, suggesting that that less TORC1 inhibition with RTB101 10 mg once daily may have greater benefit in high-risk elderly patients.

“This Phase 2b has successfully defined a dose, RTB101 10 mg once daily, to be evaluated in future pivotal studies. That dose led to a statistically significant decrease in the incidence of laboratory-confirmed RTIs and was well-tolerated in the high-risk elderly patients enrolled in the Phase 2b study. We have also identified patient populations that were particularly high responders,” said Joan Mannick, M.D., Co-Founder and Chief Medical Officer of resTORbio. “We believe the findings of this trial provide us with a clear path forward for pursuing a pivotal program for RTB101 to reduce the incidence of RTIs in high-risk elderly patients. We look forward to working closely with the U.S. Food and Drug Administration (FDA) and other regulatory agencies on this program.”

“The majority of RTIs requiring hospitalizations in the very elderly and the majority of asthma exacerbations are caused by viruses for which there are currently no approved therapies,” said Professor Sebastian Johnston, Professor of Respiratory Medicine and Allergy at the National Heart and Lung Institute, Imperial College London. “The magnitude of reduction in the incidence of laboratory-confirmed RTIs observed with RTB101 suggests that, if successfully developed and approved, RTB101 may be a new promising treatment for the very elderly and elderly patients with asthma who are at high risk of morbidity and mortality associated with RTIs.”

“The primary endpoint of this Phase 2b study, the percentage of patients with laboratory-confirmed RTIs, was chosen based on feedback from the FDA, and we look forward to discussing these results at our end of Phase 2 meeting with the agency,” said Chen Schor, Co-Founder, President and CEO of resTORbio. “RTIs are the fourth leading cause of hospitalization in patients 65 years and older, and the second leading cause of hospitalization in patients 85 years and older in the U.S. We are committed to helping the millions of elderly patients at high risk of morbidity and mortality due to RTIs.”

The Phase 2b trial was a two-part, randomized, double-blind, placebo-controlled clinical trial conducted during the winter cold and flu season in the southern hemisphere (Part 1) and northern hemisphere (Part 2). Patients enrolled were those 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 mellitus (T2DM), chronic obstructive pulmonary disease (COPD), or current smokers. The doses investigated in Part 1 were RTB101 5 mg and RTB101 10 mg once daily. The doses investigated in Part 2 were RTB101 10 mg once daily, RTB101 10 mg twice daily and RTB101 10 mg in combination with everolimus 0.1 mg once daily.

The following was observed in an analysis of the primary endpoint:

- A 30.6% decrease relative to placebo in the percentage of all patients treated with RTB101 10 mg once daily who developed one or more laboratory-confirmed RTIs ( $p=0.026$ )
- A 20.6% decrease relative to placebo in the percentage of all patients treated with RTB101 5 mg once daily who developed one or more laboratory-confirmed RTIs ( $p=0.108$ )
- No decrease relative to placebo in the percentage of patients treated with either RTB101 10 mg twice daily or the combination of RTB101 10 mg + everolimus 0.1 mg once daily who developed one or more laboratory-confirmed RTIs, suggesting that less TORC1 inhibition with RTB101 10 mg once daily may have greater benefit in high-risk elderly patients

To better understand the activity observed in the RTB101 10 mg once daily cohort, a pre-specified analysis of each patient subgroup enrolled in the study was conducted. The following decreases in the percentage of patients with laboratory-confirmed RTIs were observed in the RTB101 10 mg once daily cohort as compared to the placebo cohort:

- A 68.4% decrease in all asthma patients ( $p=0.0002$ )
- A 66.7% decrease in all patients 85 years of age and older ( $p=0.007$ )
- A 26.9% decrease in all T2DM patients ( $p=0.020$ )
- No decrease was observed in either COPD patients or current smokers; a 42.0% decrease in all patients was observed when excluding patients with COPD ( $p=0.002$ ) and a 43.9% decrease in all patients was observed when excluding current smokers ( $p=0.001$ )

All doses were observed to be well-tolerated. Data from the RTB101 10 mg once daily cohort are as follows: Adverse events (AEs) were balanced between the RTB101 10 mg once daily and placebo treatment groups. 4.5% of subjects in the RTB101 10 mg once daily cohort and 7.2% of subjects in the placebo cohort had a serious adverse event, none of which were considered related to study drug. 4.5% of subjects in the RTB101 10 mg once daily cohort and 6.1% of subjects in the placebo cohort discontinued study drug due to an AE. All AEs were mild or moderate except for 11 severe AEs in the RTB101 10 mg once daily cohort and 22 severe AEs in the placebo cohort.

This Phase 2b is the second study in which RTB101 10 mg once daily was observed to be well-tolerated and reduce the incidence of RTIs in the elderly. Together, these studies enrolled more than 900 elderly people.

#### **Conference Call and Webcast Information**

resTORbio management will host a conference call today at 8:30 a.m. ET to discuss the results of the Phase 2b trial. To participate in the conference call, please dial (877) 356-9149 (domestic) or (629) 228-0720 (international) and refer to conference ID 3181638. A live webcast of the call can be accessed in the “Investors” section of the Company’s website at [www.restorbio.com](http://www.restorbio.com). An archived webcast recording will be available on the resTORbio website beginning approximately two hours after the call.

#### **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, T2DM, 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 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 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 eight 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 ( $\geq 85$  years of age,  $\geq 65$  years of age with asthma, COPD, T2DM, or current smokers).

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

A survey was conducted by resTORbio of 100 physicians in the U.S. that treat approximately 25,000 patients aged 65 years or older monthly. Depending on their specialty, the physicians surveyed estimated that they would prescribe a therapeutic that reduced the incidence of laboratory-confirmed RTIs by 25% to approximately 30-50% of their high-risk elderly patients. Data from market surveys may not predict actual prescribing behavior should RTB101 receive regulatory approval.

### **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, as well as the intended regulatory path for our product candidates and interactions with regulatory authorities, 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; 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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resTORbio™

RTB101 Phase 2b Topline  
Data

July 25, 2018



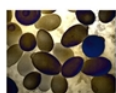
# Forward-Looking Statements

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.

# TORC1 is an evolutionarily conserved pathway that regulates aging



Yeast



Worms



Flies



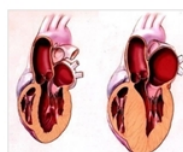
Mice



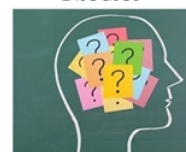
TORC1 inhibition extended lifespan and healthspan and improved the following aging-related conditions in preclinical studies:



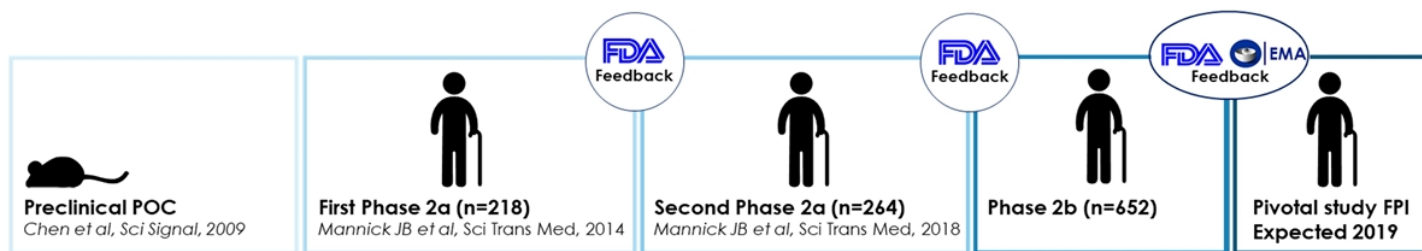
Ameliorate Heart Failure



Ameliorate Neurodegenerative Diseases



# TORC1 inhibitors improved immune function in the elderly in two previous Phase 2a clinical trials



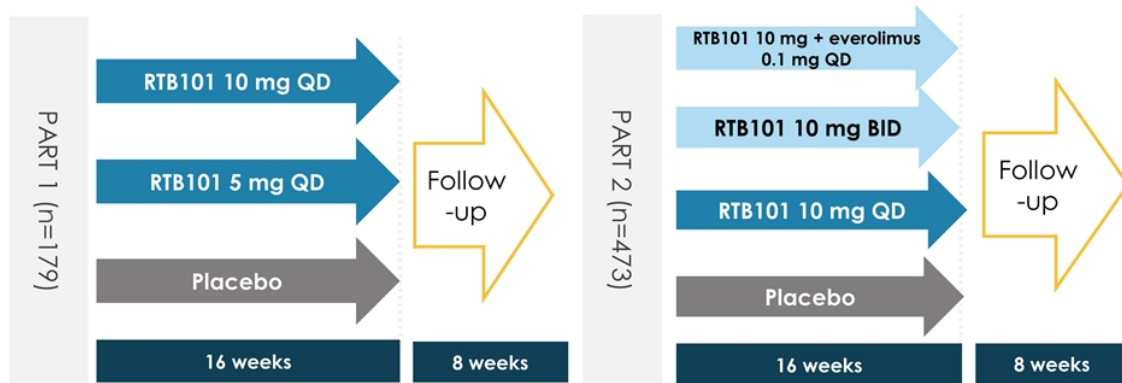
## Positive topline results in Phase 2b trial of RTB101

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- Statistically significant and clinically meaningful 30.6% reduction in the percentage of patients with one or more laboratory-confirmed respiratory tract infections (RTIs), the primary endpoint of the trial, in the RTB101 10 mg once daily cohort ( $p=0.026$ )
- Greatest reductions were observed in pre-specified analyses of asthma patients 65 years and older (68.4% reduction,  $p=0.0002$ ) and in patients 85 years and older (66.7% reduction,  $p=0.007$ )
- Successfully defined the dose and patient populations for pivotal trials:
  - Dose: RTB101 10 mg once daily
  - Patient population: 65 years or older with comorbidities, or 85 years and older
- RTB101 10 mg once daily was well-tolerated in the high-risk elderly patients enrolled in the study
- Plan to meet with regulatory authorities to discuss the design of our pivotal studies that we expect to initiate in 2019

# Phase 2b clinical trial 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 including:
  - ≥ 85 years of age
  - 65-84 years of age with one or more of the following comorbidities including:
    - Asthma
    - Chronic obstructive pulmonary disease (COPD)
    - Type 2 diabetes mellitus (T2DM)
    - Current smoker



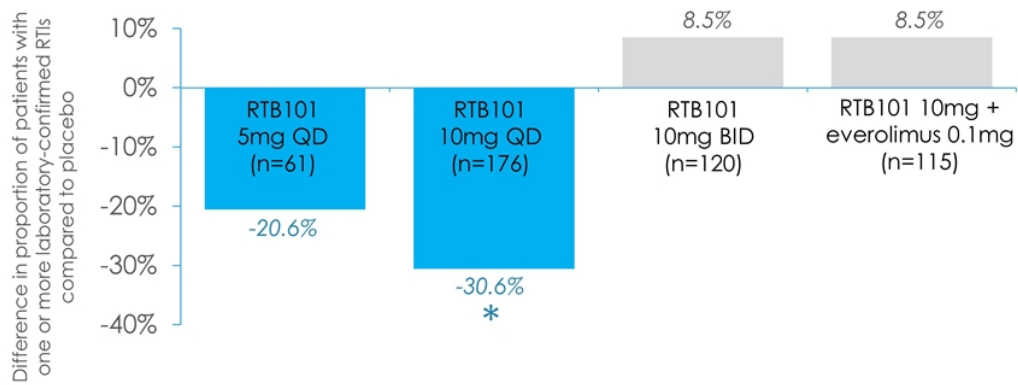
QD, once daily; BID, twice daily

# Demographics

Parameter	Statistics	Part 1				Part 2				
		RTB101 5mg (N=61)	RTB101 10mg QD <sup>1</sup> (N=58)	Placebo (N=60)	Total (N=179)	RTB101 10mg QD (N=118)	RTB101 10mg BID <sup>2</sup> (N=120)	RTB101 + everolimus (N=115)	Placebo (N=120)	Total (N=473)
Age at Randomization (Year)	n	61	58	60	179	118	120	115	120	473
	Mean (SD)	74.0 (8.2)	76.5 (7.9)	74.4 (7.3)	74.9 (7.9)	73.1 (6.9)	73.0 (6.9)	73.9 (7.0)	73.2 (7.2)	73.3 (7.0)
Sex, n (%)	Male	33 (54.1)	31 (53.4)	36 (60.0)	100 (55.9)	52 (44.1)	62 (51.7)	58 (50.4)	53 (44.2)	225 (47.6)
	Female	28 (45.9)	27 (46.6)	24 (40.0)	79 (44.1)	66 (55.9)	58 (48.3)	57 (49.6)	67 (55.8)	248 (52.4)
Race, n (%)	White	56 (91.8)	54 (93.1)	57 (95.0)	167 (93.3)	114 (96.6)	110 (91.7)	106 (92.2)	109 (90.8)	439 (92.8)
	Black or African American	0	0	0	0	4 (3.4)	9 (7.5)	5 (4.3)	10 (8.3)	28 (5.9)
	Asian	2 (3.3)	2 (3.4)	0	4 (2.2)	0	0	0	0	0
	American Indian or Alaska Native	0	0	0	0	0	1 (0.8)	1 (0.9)	0	2 (0.4)
	Native Hawaiian, Maori or Other Pacific Islander	0	0	0	0	0	0	0	0	0
	Other	3 (4.9)	2 (3.4)	3 (5.0)	8 (4.5)	0	0	3 (2.6)	1 (0.8)	4 (0.8)
	Not reported	0	0	0	0	0	0	0	0	0
Ethnicity, n(%)	Not Hispanic or Latino	61 (100.0)	58 (100.0)	60 (100.0)	179 (100.0)	108 (91.5)	114 (95.0)	101 (87.8)	108 (90.0)	431 (91.1)
	Hispanic or Latino	0	0	0	0	10 (8.5)	6 (5.0)	14 (12.2)	12 (10.0)	42 (8.9)
	Not reported	0	0	0	0	0	0	0	0	0

<sup>1</sup> QD, once daily; <sup>2</sup> BID, twice daily

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

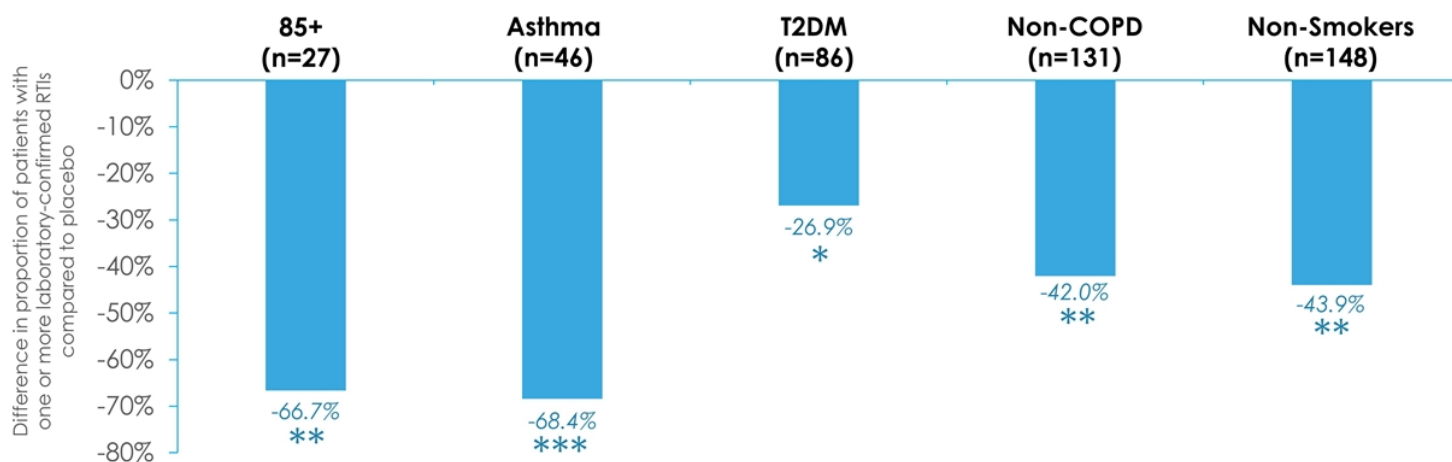


<b>P-value<sup>1</sup></b>	<b>0.108</b>	<b>0.026</b>	<b>0.617</b>	<b>0.694</b>
<b>Odds ratio<sup>2</sup> (CI<sup>3</sup>)</b>	0.615 (0.323; 1.174)	0.604 (0.394; 0.927)	1.100 (0.650; 1.860)	1.180 (0.689; 2.022)

<sup>1</sup>One-sided p-value; <sup>2</sup>Odds ratio represents the odds of experiencing one or more laboratory confirmed RTIs in the active treatment group versus the placebo group; <sup>3</sup>90% confidence interval; \*p<0.05, considered to be statistically significant



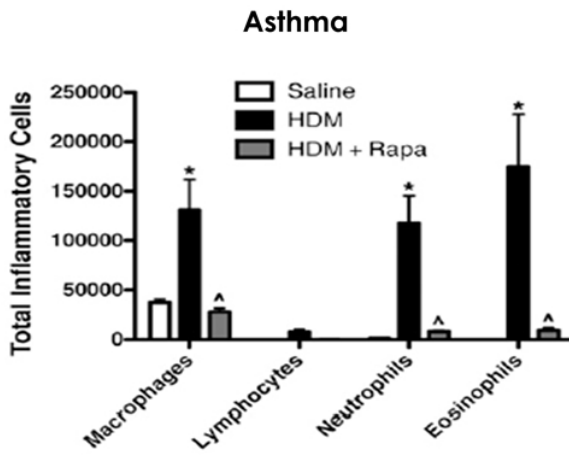
# Pre-specified analyses of laboratory-confirmed RTI reduction in patient subgroups treated with RTB101 10 mg once daily



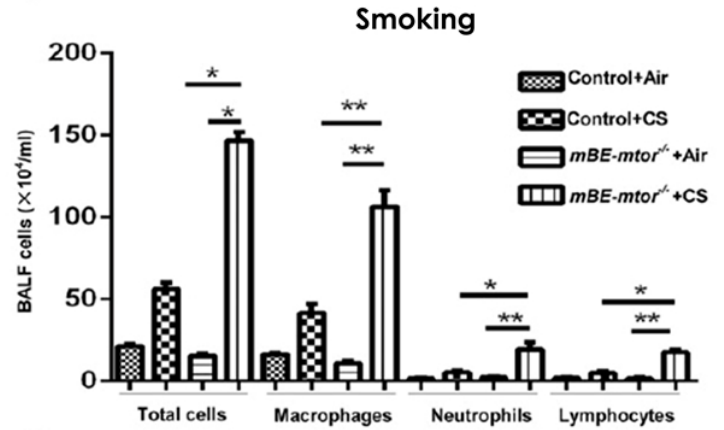
<b>P-value<sup>1</sup></b>	<b>0.007</b>	<b>0.0002</b>	<b>0.020</b>	<b>0.002</b>	<b>0.001</b>
<b>Odds ratio<sup>2</sup> (CI<sup>3</sup>)</b>	0.182 (0.059; 0.564)	0.110 (0.040; 0.305)	0.337 (0.141; 0.806)	0.317 (0.162; 0.619)	0.309 (0.165; 0.577)

<sup>1</sup>One-sided p-value; <sup>2</sup>Odds ratio represents the odds of experiencing one or more laboratory confirmed RTIs in the active treatment group versus the placebo group; <sup>3</sup>90% confidence interval; \*p<0.05, \*\*p<0.01, \*\*\*p<0.001

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



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)<sup>1</sup>



Disruption of mTOR selectively in bronchial epithelial cells (mBE-mtor<sup>-/-</sup>) significantly **increased** cigarette smoke (CS)-induced lung inflammation in a COPD model in which mice were exposed to cigarette smoke for 6 months<sup>2</sup>

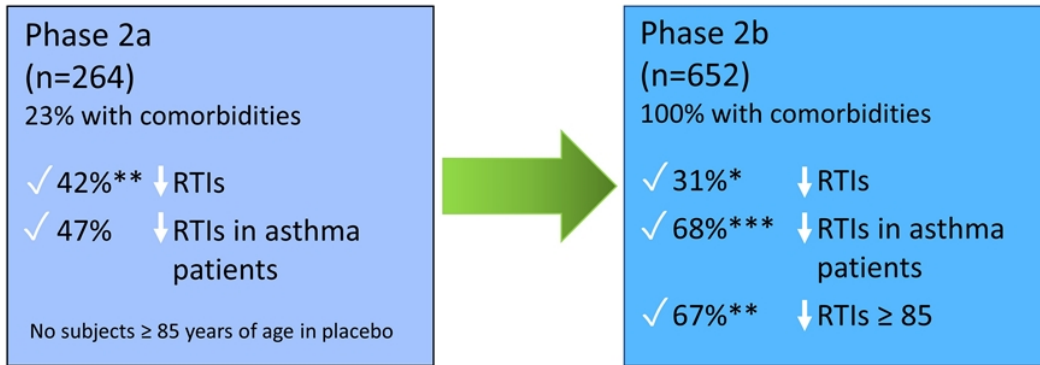
<sup>1</sup>Mushaben E. M. et al., *J Immunol* 2011;187:5756-5763; <sup>2</sup> Wang Y et al., *J Immunol* 2018;200:2571-2580; \*p<0.05, \*\*p<0.01

# Safety and tolerability

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- Adverse events (AEs) were balanced between the RTB101 10 mg once daily and placebo cohorts
- 1 unrelated death occurred in the RTB101 10 mg once daily cohort (patient was hit by car while riding a bicycle), 1 unrelated death occurred in the placebo cohort (unknown cause)
- 4.5% of subjects in the RTB101 10 mg once daily cohort and 7.2% of subjects in the placebo cohort had a serious adverse event, none of which were considered related to study drug
- 4.5% of subjects in the RTB101 10 mg once daily cohort and 6.1% of subjects in the placebo cohort discontinued study drug due to an AE
- All AEs were mild or moderate in severity except for 11 severe AEs in RTB101 10 mg once daily cohort and 22 severe AEs in the placebo cohort

# Consistent efficacy of RTB101 10 mg once daily observed in two Phase 2 clinical trials enrolling more than 900 elderly people



\*p<0.05, \*\*p<0.01, \*\*\*p<0.001

## Summary of 16-week analysis of Phase 2b

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- There was a statistically significant and clinically meaningful reduction in the percentage of patients with one or more laboratory-confirmed RTIs, the primary endpoint of the study, in the RTB101 10 mg once daily cohort
- RTB101 10 mg twice daily and RTB101 10 mg in combination with everolimus 0.1 mg did not meet the primary endpoint, suggesting that less TORC1 inhibition with RTB101 10 mg once daily may have greater benefit in high-risk elderly patients
- Study successfully defined the dose and patient populations to include in our pivotal trials:
  - RTB101 10 mg once daily
  - 65 years and older non-smokers, 65 years and older with asthma, or 85 years and older
- RTB101 10 mg once daily was well-tolerated in the high-risk elderly patients enrolled in the Phase 2b study
- Plan to meet with regulatory authorities to discuss design of pivotal trials and initiate pivotal trials in 2019

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# Medical Need & Market Opportunity

## RTIs represent a significant healthcare burden

- RTIs are the 4<sup>th</sup> most common cause for hospitalization in 65+<sup>1</sup> (2<sup>nd</sup> in 85+<sup>1</sup>)
- RTIs are the 7<sup>th</sup> leading cause of death in 65+<sup>2</sup> (5<sup>th</sup> in 85+<sup>2</sup>)
- RTIs are the leading cause of asthma exacerbations<sup>3</sup>
- The majority of RTIs are caused by viruses for which there are no approved therapies<sup>4</sup>
- Decreasing the incidence of RTIs in the elderly may significantly decrease health care costs



## Estimated 75 million elderly people at increased risk of RTI-related morbidity and mortality in the U.S., major European countries and Japan

	 <b>US</b>	 <b>EU5</b>	 <b>JP</b>
<b>Elderly people (65-74 years old):</b> <i>With comorbidities (COPD, asthma, T2DM, CHF)</i>	11M	13M	7M
<b>Elderly people (75-84 years old):</b> <i>With comorbidities (COPD, asthma, T2DM, CHF)</i>	7M	11M	6M
<b>Elderly people (85+ years old):</b>	6M	9M	5M
<b># Elderly People (2016)</b>	<b>24M</b>	<b>33M</b>	<b>18M</b>
<b>Average Annual Growth Rate</b>	3%	2%	1%

Represents 2016 figures

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16



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

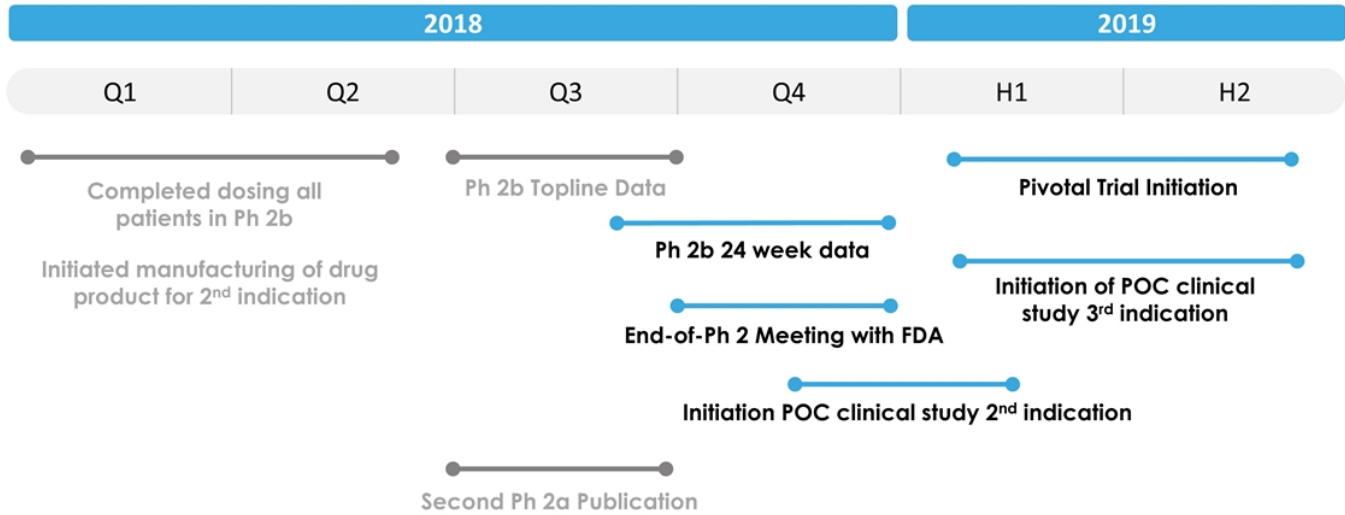
## Physician survey\*: Expected use in target populations

% Reduction in RTI	Estimated % prescribed in aged 85+ or 65+ with comorbidities, patient-weighted
25%	>30%
33%	>40%
40%	>45%

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

Medical Specialty		Practice characteristics	
Geriatrics	25	Years practicing medicine	Avg 19 (median 19.5, range 6-33)
Primary Care	50	# pts ≥ 65 seen/month	Avg 250 (median 220, range 80-600)
Pulmonologist	25	% services billed to Medicare	Avg 63% (median 65%, range 30-100%)

# Near term planned clinical milestones and path forward



POC = proof of concept



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RTB101 Phase 2b Data Review

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