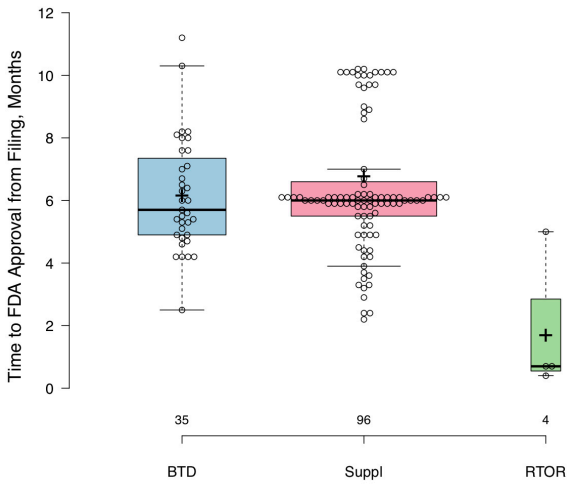
FDA's Real-time Oncology Review Pilot Program Substantially Reduces the Time to FDA Approval from Filing – Initial Results from Analysis of Blomquist & Associates' LTTA Oncology Database.

BOULDER, Colorado — January 8, 2019 — Blomquist & Associates today announced the initial results of its study evaluating the impact of the FDA's Real-time Oncology Review Pilot Program (RTOR) on the Time to FDA Approval (TTA) from filing for investigational oncology drugs. The initial results showed an **88% reduction in the median TTA from Filing** for indications approved under RTOR. The median TTA for agents approved under RTOR was 0.7 months compared to 5.7 or 6.0 months for approvals of agents with Breakthrough Therapy Designation (BTD) or supplemental indication approvals (suppl), respectively. The differences in median TTA were not statistically significant (p=0.10), as there was a small RTOR sample size (only four indications approved to date). However, there was a trend toward significance. For financial forecasting, the substantial reduction in median TTA from Filing should be considered when forecasting TTA for those agents which qualify for RTOR. Summary results of the study are shown in the Graph and Table, below.

Further, the initial results showed the Likelihood Time to FDA Approval (LTTA) was 75% at two months from Filing for indications approved under RTOR, significantly higher compared to 0% at two months either for approvals of agents with BTD or supplemental indication approvals. Summary results of the study are shown in the Graph and Table, below.

Graph: Time to FDA Approval from Filing



Legend: BTD = agent approvals with Breakthrough Therapy Designation (shown in blue). Suppl = Supplemental indication approvals (shown in pink). RTOR = agents approved under Real-time Oncology Review (shown in green). Blue = BTD; Pink = Suppl; Green = RTOR. Box Plot description: Center lines show the medians; box limits indicate the 25th and 75th percentiles as determined by R software; whiskers extend 1.5 times the interquartile range from the 25th and 75th percentiles; crosses represent sample means; width of the boxes is proportional to the square root of the sample size; data points are plotted as open circles. The graph shows the differences in TTA from Filing for indications approved under RTOR versus approvals for agents with BTD or supplemental indication approvals.

Table: Summary Statistics for Time to FDA Approval from Filing

Group	BTD	Suppl.	RTOR
Sample size	35	96	4
TTA – Time to FDA Approval			
Mean (months)	6.16	6.78	1.70
Upper whisker	10.30	7.00	5.00
3rd quartile	7.35	6.60	2.85
Median (months)	5.70	6.00	0.70
1st quartile	4.90	5.50	0.55
Lower whisker	2.50	3.90	0.40
Mood's Median Test	4.63, p = 0.10		
LTTA – Likelihood Time to FDA Approval			
at 2 months	0%	0%	**75%**
at 4 months	2.9%	11.5%	75%
at 6 months	57.1%	70.8%	100%
at 8 months	85.7%	77.1%	100%
at 10 months	94.3%	95.8%	100%

The initial results demonstrate that the FDA's RTOR Pilot Program delivers a substantial reduction in TTA from Filing for those agents that qualify for the RTOR Pilot Program.

Blomquist & Associates' study compared the TTA (Time to FDA Approval) from Filing, and the LTTA (Likelihood time to FDA approval from Filing) for the four indications approved to date under RTOR, compared to 35 approved agents granted Breakthrough Therapy Designation (first FDA approval; non-RTOR), and compared to 96 supplemental indication approvals (non-RTOR) for oncology agents in the LTTA Oncology Database.

Data used for this study were extracted from Blomquist & Associates' LTTA Oncology Database Version 1.46, covering FDA, EC and MHLW approvals for investigational oncology drugs. At the time the study was conducted, the LTTA Oncology Database tracked >90 oncology drugs and >250 investigational oncology indications.

Previously, Blomquist & Associates announced the first study of Likelihood Time to Regulatory Approval for Investigational Oncology Drugs in the U.S. and Europe on April 26, 2018. This study of the impact of FDA's RTOR Pilot Program on TTA from Filing builds on the earlier work and demonstrates the power and timeliness of analysis of the LTTA Oncology Database.

ABOUT FDA's REAL-TIME ONCOLOGY REVIEW PILOT PROGRAM:

The U.S. Food and Drug Administration states the aims for the agency's Real-Time Oncology Review (RTOR) Pilot Program are "…to make the development and review of cancer drugs more efficient, while improving FDA's rigorous standard for evaluating efficacy and safety. With [the] real-time review, the FDA [starts] evaluating the clinical data as soon as the trial results become available, enabling FDA to be ready to approve the new indication upon filing of a formal application with the Agency." Effectively, FDA is taking steps to streamline and accelerate the approval process for certain cancer drugs in clinical development. The FDA states the scope of the pilot program is for "…supplemental New Drug Application (NDA) and supplemental Biologic License Application (BLA) submissions…", "Drugs likely to demonstrate substantial improvements over available therapy…[and] may include drugs previously granted Breakthrough Therapy Designation for the same or other indications…" The RTOR Pilot Program was announced by U.S. Food and Drug Commissioner Scott Gottlieb on June 2, 2018 during the American Society of Clinical Oncology annual meeting.

ABOUT LTTA ONCOLOGY DATABASE

The LTTA Oncology Database tracks clinical development and regulatory approvals of investigational oncology drugs to assess the TTA and LTTA by U.S. FDA, EC (European Commission) and MHLW (Japan Ministry of Health, Labour and Welfare). Presently, the LTTA Oncology Database is available on a consulting basis from Blomquist & Associates. A portal / dashboard to access the LTTA Oncology Database is under development and is planned to be available on a subscription basis in the future. The Database is populated on a regular basis with updated information from clinical trial records, medical meetings, company press releases, corporate earnings reports and presentations, regulatory filing and approval records, and a broad range of other sources. Where possible, links to the various data sources utilized in creating the specific data records are included in the database at the time of data entry to enable review and validation of data sources. Additional LTTA databases are under development for metabolic, neurology and orphan drugs.

When creating risk-based financial forecasts for investigational drugs, there is a need for data about the time to approval from key events and the likelihood (probability) time to approval by certain times from key events. Analysis of Blomquist & Associates' LTTA Oncology Database fills this need for investigational oncology drugs.

ABOUT BLOMQUIST & ASSOCIATES

Blomquist & Associates is a boutique consulting firm in Life Sciences. We advise private equity investors, as well as business executives in the pharma and biotech industries. We employ an evidence-based and data-driven approach to provide actionable information that helps our clients make confident decisions on valuation, forecasting and investments. We offer a broad range of consulting services, including around TTA, LTTA, risk-based forecasting and competitive analysis.

CONTACT

To learn more about this study, the LTTA Oncology database, or our other services, please contact:
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REFERENCES

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FDA Real-time Oncology Review Pilot Program, last updated 10/15/2018
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