May 9, 2017

Novelion Therapeutics Reports First Quarter 2017 Financial Results

- *Global Expansion Plans for Metreleptin Opportunity to be Announced this Summer*
- *Defined Clinical Development Plan for Zuretinol Following Positive FDA Meeting*
- *Company Reiterates FY 2017 Total Net Revenues Guidance Between $155 and $165 million*
- *Conference call to be held today at 8:30 a.m. ET*

VANCOUVER, British Columbia, May 09, 2017 (GLOBE NEWSWIRE) -- Novelion Therapeutics Inc. (NASDAQ:NVLN), a biopharmaceutical company dedicated to developing and commercializing innovative new therapies for individuals living with rare and unmet need diseases ("Novelion" or the "Company"), today reported preliminary financial results for the first quarter ended March 31, 2017 and provided an overview of recent business highlights, including an update on its clinical development programs for metreleptin and zuretinol.

"Our execution to transform Novelion into a growth company and a leading orphan and rare disease company continued during the first quarter," said Chief Executive Officer Mary Szela. "We are focused on maximizing the value of our commercial assets, and expanding the potential of metreleptin, a potentially transformational therapy for a range of low-leptin mediated metabolic diseases. Operationally, significant progress has been made to reduce expenses and enhance efficiencies globally and cross-functionally. We also further strengthened our commercial business by implementing a new commercial structure in the U.S. that we believe is better aligned to effectively address the needs of physicians and payors, and therefore enable us to better serve the needs of adult HoFH and GL patients."

Ms. Szela continued, "We plan to unveil our strategy to maximize the clinical and commercial value of metreleptin across a broad range of low-leptin mediated metabolic diseases this summer. Following a very productive meeting with the U.S. Food and Drug Administration (FDA), we believe we have delineated a clear regulatory path for the clinical development of our differentiated drug candidate for rare ophthalmic diseases, zuretinol, another important asset in our pipeline."

Novelion plans to focus development efforts on a pediatric population and believes zuretinol will qualify for a pediatric priority review voucher. The Company estimates that if approved for the treatment of inherited retinol disease caused by underlying mutations in retinal pigment epithelium protein 65 (RPE65) and lecithin retinol acyltransferase (LRAT) genes, zuretinol could achieve approximately $200 million in peak sales.

First Quarter 2017 Highlights & Business Update

- Deployment, and completion of training, of a new U.S. commercial sales force in the first quarter, to drive expected revenue growth for **MYALEPT®** and **JUXTAPID**®.
- **MYALEPT**: Novelion reported net revenues of MYALEPT of $14.0 million in the first quarter of 2017, $11.5 million, or 82 percent, of which were from prescriptions written in the U.S.
- **JUXTAPID**: Novelion reported net revenues of JUXTAPID of $16.0 million in the first quarter of 2017, $10.9 million, or 68 percent, of which were from prescriptions written in the U.S.
- Novelion reported total net revenues of $30.0 million in the first quarter of 2017. First quarter sales were impacted by the implementation of a new commercial infrastructure as well as seasonal effects pertaining to insurance renewals. The Company expects continued steady growth in revenues throughout the year.
- Novelion ended the first quarter of 2017 with $84.8 million in unrestricted cash. Cash usage was driven by net loss, a reduction of payables and accrued expenses, as well as restructuring and other nonrecurring payments that were specific to the first quarter.
- On March 31, 2017, Novelion announced the appointment of Mark Corrigan, M.D. to its board of directors.
- Novelion has undertaken an evaluation process to prioritize and pursue potential life cycle management, and research and development opportunities, for metreleptin. The Company plans to provide a full update on these plans this summer.

Recent Clinical Data Presentations for Metreleptin and Zuretinol

- At the Endocrine Society's 99th Annual Meeting and Expo, ENDO 2017, held April 1-4, 2017, in Orlando, Florida, four separate data presentations by academic researchers investigating metreleptin (which is only approved to treat generalized lipodystrophy) and lipodystrophy-related diseases were presented. One was an open-label study in 23 patients with partial lipodystrophy-associated NASH, in which the study team (at Michigan Medical) reported that patients with a lower baseline leptin level appeared to have a higher response rate after one year of treatment with metreleptin. Furthermore, a statistically significant improvement in NASH scores over baseline was found in 18 patients who completed treatment at one year. The most frequently reported adverse events in the study occurring in more than 20 percent of patients were: upper respiratory infections, hypoglycemia, and diarrhea.

- At the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting held May 7-11, 2017, in Baltimore, MD, a retrospective, multi-center, case history study entitled, "Natural History of Inherited Retinal Disease (IRD) in Patients with Mutations in the RPE65 or LRAT Genes" was presented. Twenty five patients who received a single 7-day oral course of treatment with zuretinol showed a statistically significant improvement in Visual Field, or VF, and best-corrected Visual Acuity, or BCVA, during the 30 days following treatment compared to twenty nine untreated patients who showed statistically significant losses in VF and BCVA area over time. Adverse events for zuretinol are consistent with the retinoid class of medications. Headache and fatigue are the most common treatment-related adverse events followed by erythema, nausea, photophobia, photopsia, flushing and vomiting. Reversible elevations in triglyceride, LDL, cholesterol, AST and ALT levels and reduction in HDL and thyroxine have been recorded. One serious adverse drug reaction (pseudotumor cerebri or intracranial hypertension, a known class effect of retinoids) has been reported and was resolved.

2017 Financial Guidance

The Company reiterated the following net revenues financial guidance for full year 2017:

- Total net revenues between $155 million and $165 million;
- MYALEPT net revenues between $75 million and $80 million; and
- JUXTAPID net revenues between $80 million and $85 million.

The Company intends to provide full year 2017 operating expense guidance upon conclusion of its ongoing R&D program review and prioritization this summer. As part of this review, Novelion continues to identify and implement cost-reduction initiatives.

First Quarter 2017 Financial Results

On November 29, 2016, the Company completed its acquisition of Aegerion Pharmaceuticals, Inc. ("Aegerion"), which develops and commercializes MYALEPT and JUXTAPID. The acquisition has been accounted for as a business combination in which Novelion was considered the acquirer of Aegerion. As such, under U.S. Generally Accepted Accounting Principles ("GAAP"), the financial statements of Novelion are treated as the historical financial statements of the consolidated companies, with the results of Aegerion being included from November 29, 2016. This release also includes pro forma adjusted non-GAAP financial information showing pro forma results of operations of Novelion as if the acquisition had occurred on January 1, 2016. Reconciliation of the financial results on a GAAP versus non-GAAP basis are provided below the financial information that follows.

GAAP total net revenues for the first quarter of 2017 were $30.0 million compared to the prior year's first quarter revenues of $0 million. GAAP net revenues for MYALEPT in the first quarter of 2017 were $14.0 million compared to $0 for the same period in 2016. GAAP net revenues for JUXTAPID in the first quarter of 2017 were $16.0 million compared to $0 in the prior year.

Pro forma total net revenues for the first quarter of 2017 were $30.0 million. Pro forma net revenues for MYALEPT in the first quarter of 2017 were $14.0 million compared to $9.5 million for the same period in 2016. Pro forma net revenues for JUXTAPID in the first quarter of 2017 were $16.0 million compared to $26.2 million for the same period in 2016.

GAAP total operating expenses for the first quarter of 2017 were $35.2 million compared to total operating expenses of $8.9 million for the same period in 2016. GAAP SG&A expenses were $24.5 million in the first quarter of 2017 compared to $5.9 million for the same period in 2016. GAAP R&D expenses were $9.3 million in the first quarter of 2017 compared to $3.0 million for the same period in 2016.

During the first quarter of 2017, SG&A expenses on a pro forma basis were $23.0 million compared to $42.0 million for the same period in 2016. The decrease in pro forma SG&A expenses in the first quarter of 2017 compared with the same period in 2016 was primarily related to a reduction in headcount and legal and banking fees that occurred in the first quarter of 2016.

R&D expenses on a pro forma basis were $9.0 million compared to $12.5 million for the same period in 2016. The decrease in R&D expenses in the first quarter of 2017 compared with the same period in 2016 was primarily related to a reduction in headcount and decreased contract manufacturing and clinical trial expenses due to the timing of R&D activities.

GAAP net loss in the first quarter of 2017 was $31.0 million compared to GAAP net loss of $21.9 million during the same period in 2016.

Net loss on a pro forma basis in the first quarter of 2017 was $8.7 million, compared to $71.0 million for the same period in 2016.

As of March 31, 2017, the Company's consolidated unrestricted cash balance was $84.8 million compared to $108.9 million at December 31, 2016. As of March 31, 2017, there were 18,558,072 shares outstanding. At March 31, 2017, total debt was $325 million, reflecting the principal amount of convertible debt issued by Aegerion and consolidated as a result of the acquisition.

Conference Call Details

Novelion will hold a conference call to discuss its financial results, business highlights and outlook today, May 9, 2017 at 8:30 a.m. ET. In addition, the Company will answer questions concerning business and financial developments and other matters affecting the Company.

To listen to the conference call, dial (866) 516-3002; international callers dial (760) 298-5082. In addition, the presentation will be webcast live, and may be accessed for up to 90 days following the call, by visiting the "Investors" section of Novelion's website, www.novelion.com. An accompanying slide presentation also can be accessed via the "Investors" section of the website.

About Novelion Therapeutics

Novelion Therapeutics is a biopharmaceutical company dedicated to developing new standards of care for individuals living with rare diseases. Novelion has a diversified commercial portfolio through its indirect subsidiary, Aegerion Pharmaceuticals, Inc., and is also developing zuretinol acetate for the potential treatment of inherited retinal disease caused by underlying mutations in RPE65 or LRAT genes. The company seeks to advance its portfolio of rare disease therapies by investing in science and clinical development.

Non-GAAP Results

The non-GAAP results in this press release, including, without limitation, non-GAAP net revenues, non-GAAP operating expenses, non-GAAP R&D expenses and non-GAAP SG&A expenses and non-GAAP net loss, are provided as a complement to results provided in accordance with GAAP because management believes, when considered together with the GAAP information, these non-GAAP financial measures help indicate underlying trends in the Company's business, are important in comparing current results with prior period results and provide additional information regarding the Company's financial performance. In particular, management believes that the pro-forma financial information facilitates the evaluation of the impact of Novelion's acquisition of Aegerion on the business and performance of the Company. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally, and to manage the Company's business and evaluate its performance. The non-GAAP financial measures have no standardized meaning under GAAP and therefore may not be comparable to similar measures presented by other companies. The non-GAAP financial measures are not intended to be considered in isolation or as a substitute for, or superior to, the financial measures prepared and presented in accordance with GAAP and should be reviewed in conjunction with the relevant GAAP financial measures. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial information.

Forward-Looking Statements

Certain statements in this press release constitute "forward-looking statements" of Novelion within the meaning of applicable laws and regulations and constitute "forward-looking information" within the meaning of applicable Canadian securities laws, including statements regarding expectations such as growth in net revenues, expectations about 2017 revenues, planned regulatory filings, approvals and activities, maximizing the value of metreleptin, planned business development activities, drug development, marketing authorizations and label expansions, as well as long-term growth prospects. Forward-looking statements are based on estimates and assumptions made by Novelion in light of current conditions and expected future developments, as well as other factors that Novelion believes are appropriate in the circumstances, including, but not limited to, our financial position and execution of our business strategy, post-acquisition synergies, resolution of litigation and

investigations, receipt of regulatory approvals, and product competition, market acceptance, sales, pricing, reimbursement and side effects. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond our control, which could cause actual results to differ materially from those contemplated in these forward-looking statements. Many such risks, uncertainties and other factors are taken into account as part of our assumptions underlying these forward-looking statements and include, among others, the following: the possibility that the anticipated benefits and synergies from Novelion's acquisition of Aegerion cannot be fully realized or may take longer to realize than expected; the possibility that costs or difficulties related to the integration of Aegerion and Novelion operations will be greater than expected; the risk that market acceptance of JUXTAPID and MYALEPT in the U.S. may not continue at the levels we expect, and may be lower outside the U.S., including in Brazil and Japan, than we expect; the risk that the conversion of prescriptions for JUXTAPID or MYALEPT into patients on therapy may be lower than we expect or the drop-out rate may be higher than we expect; the risk that the prevalence of the diseases JUXTAPID and MYALEPT treat, or that we are pursuing treatment for, may be lower than we estimate, and that it may be more difficult to identify patients than we expect; the risk that the side effect profile or other results for JUXTAPID and MYALEPT in commercial use and in further clinical studies are inconsistent, in scope and severity, with the side effect profile and other results observed in the pivotal study of each drug; the risk that the negative impact of PCSK9 inhibitors on JUXTAPID sales will be greater than we currently expect, particularly in the U.S., where the negative impact has been greater than we expected to date, or that other competitive products will negatively impact our results; the risk that private or government payers may refuse to reimburse Aegerion's or our products, or may impose onerous restrictions that hinder reimbursement or significantly limit or cap the price Aegerion or we charge or the number of reimbursed patients who receive products; the risk that revisions to the JUXTAPID Risk Evaluation and Mitigation Strategies (REMS) Program, and the implementation of the revised REMS Program, may negatively impact U.S. sales; the risk that net revenues for MYALEPT in the U.S. may be negatively impacted if there are more Medicaid patients prescribed MYALEPT than we expect; the risk that net revenues for JUXTAPID in the U.S. may be negatively impacted by Medicare patients not being able to afford JUXTAPID; the risk that named patient sales for JUXTAPID and MYALEPT in Brazil and other key countries outside the U.S. may not be at the levels we expect; the risk that regulatory authorities in regions or countries where JUXTAPID or MYALEPT is not yet approved may refuse to approve such products or that regulatory authorities may refuse to approve additional indications for such products, such approvals are not made on a timely basis or such approvals impose significant restrictions or require additional development; the risk that exchange rates will negatively impact the amount of net revenues recognized; the risk that the initiation of future clinical trials may be delayed or that larger or a greater number of clinical trials necessary to obtain approvals of indications for our products may be required; the risk that we will not be successful in our lifecycle management or business development efforts; the risk that Aegerion's and our patent portfolios and marketing and data exclusivity may not be as strong as we anticipate; the risk of unexpected manufacturing issues affecting future commercial or clinical supply; the risk that Aegerion incurs more costs than we expect in responding to investigations, defending litigation and resolving litigation; the risk that any of the foregoing may cause net revenues to be lower than we expect, or that we may incur unanticipated expenses in connection with our activities; the risk that we may not be able to successfully execute strategic plans, including our cost-reduction program; and the other risks inherent in the commercialization, drug development and regulatory approval process; the risk associated with our ability to be granted a Rare Pediatric Disease Designation and any subsequent qualification for a Rare Pediatric Disease Priority Review Voucher, including the risk that zuretinol will not qualify under the current or any future applicable criteria for designation as a Rare Pediatric Disease or that an NDA for zuretinol will not qualify for a Priority Review Voucher, and the risk that future changes to the zuretinol program and/or the Voucher Program, including related to the transferability of the Priority Review Voucher, limit the future benefits of the Rare Pediatric Disease Designation and/or Priority Review Voucher. The terms of Aegerion's agreement in principle related to its class action litigation include risks related to the final approval by the court of the final settlement terms, including that the payment amount and availability of insurance could be amended and the amount and terms of any final settlement may be substantially higher and less favorable than we anticipate based on the terms of the preliminary agreement in principle, and the possibility that the court may materially alter or fail to approve the settlement terms. In addition, Aegerion's agreement in principle with the U.S. Department of Justice ("DOJ") and the U.S. Securities and Exchange Commission ("SEC") relating to the investigations by these agencies and the terms of potential final settlements with these agencies include risks associated with the required approvals of final settlement terms by relevant government agencies, such as the proposed settlement with the DOJ being subject to approval of supervisory personnel within the DOJ and relevant federal and state agencies and approval by a U.S. District Court judge of the criminal plea and sentence and the civil settlement agreement, and the proposed settlement with the SEC being subject to review by other groups in the SEC and approval by the Commissioners of the SEC. The terms of the preliminary agreements in principle may change following further negotiations. The amount and terms of any final settlement may be substantially higher and less favorable than we anticipate based on the terms of the preliminary agreements in principle. Final settlement terms could include the imposition of additional penalties, further limiting Aegerion's ability to conduct its business as currently conducted and as planned to be conducted. Additionally, the DOJ and the SEC each likely will outline their views of the factual background in connection with any final settlement. The government's recitation of their assessment of the background could lead to additional legal claims or investigations by state government entities or private parties and may have adverse effects on Aegerion's existing class action litigation, including the agreement in principle to settle such litigation, commercial operations and contracts.

This press release also contains "forward-looking information" that constitutes "financial outlooks" within the meaning of applicable Canadian securities laws. This information is provided to give investors general guidance on management's current expectations of certain factors affecting our business, including our financial results. Given the uncertainties,

assumptions and risk factors associated with this type of information, including those described above, investors are cautioned that the information may not be an appropriate subject of reliance for other purposes.

For additional disclosure regarding these and other risks we face, see the disclosure contained in the "Risk Factors" section of Novelion's Annual Report on Form 10-K filed on March 30, 2017, available on the SEC's website at www.sec.gov. Except as required by law, we undertake no obligation to update or revise the information contained in this press release, whether as a result of new information, future events or circumstances or otherwise.

Investors and others should note that we communicate with our investors and the public using our company website www.novelion.com, including, but not limited to, company disclosures, investor presentations and FAQs, SEC filings, press releases, public conference calls transcripts and webcast transcripts. The information that we post on these websites could be deemed to be material information. As a result, we encourage investors, the media and others interested to review the information that we post there on a regular basis. The contents of our website shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

U.S. INDICATIONS AND IMPORTANT SAFETY INFORMATION

MYALEPT® (metreleptin) for injection is a leptin analog indicated as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy. LIMITATIONS OF USE: The safety and effectiveness of MYALEPT for the treatment of complications of partial lipodystrophy or for the treatment of liver disease, including nonalcoholic steatohepatitis (NASH), have not been established.

Anti-metreleptin antibodies with neutralizing activity have been identified in patients treated with MYALEPT. T-cell lymphoma has been reported in patients with acquired generalized lipodystrophy, both treated and not treated with MYALEPT. For more detailed information, please see additional Important Safety Information and the Prescribing Information for MYALEPT.

JUXTAPID® (lomitapide) capsules is a microsomal triglyceride transfer protein inhibitor indicated as an adjunct to a low-fat diet and other lipid-lowering treatments, including low-density lipoprotein (LDL) apheresis where available, to reduce LDL cholesterol, total cholesterol, apolipoprotein B, and non-high-density lipoprotein cholesterol in patients with homozygous familial hypercholesterolemia (HoFH). LIMITATIONS OF USE: The safety and effectiveness of JUXTAPID have not been established in patients with hypercholesterolemia who do not have HoFH, including those with heterozygous familial hypercholesterolemia (HeFH). The effect of JUXTAPID on cardiovascular morbidity and mortality has not been determined.

JUXTAPID can cause elevations in transaminases, as well as increases in hepatic fat, with or without concomitant increases in transaminases. Because of the risk of hepatotoxicity, JUXTAPID is available only through a restricted distribution program called the JUXTAPID REMS PROGRAM. For more detailed information, please see additional Important Safety Information and the Prescribing Information for JUXTAPID.

Novelion Therapeutics Inc.

Condensed Consolidated Statements of Operations

(unaudited)

	Three Months Ended March 31,	
(In 000s)	**2017**	**2016**
Net revenues	$ 29,984	$ —
Cost of product sales	16,445	—
Operating expenses:		
Selling, general and administrative	24,451	5,936
Research and development	9,300	2,990
Restructuring charges	1,451	—
Total operating expenses	35,202	8,926
Loss from operations	(21,663)	(8,926)
Interest (expense) income, net	(9,212)	75
Fair value loss on investment	—	(12,960)
Other income (expense), net	52	(77)
Loss before provision for income taxes	**(30,823)**	**(21,888)**
Provision for income taxes	(139)	(6)

Net loss	**$ (30,962)**	**$ (21,894)**
Net loss per common share—basic and diluted	$ (1.67)	$ (2.07)
Weighted-average shares outstanding—basic and diluted	18,540	10,565

Novelion Therapeutics Inc.

Condensed Consolidated Balance Sheets

(unaudited)

(In 000s)	**March 31, 2017**	**December 31, 2016**
Cash and cash equivalents	$ 84,756	$ 108,927
Restricted cash	240	390
Accounts receivable, net	11,439	9,339
Inventories	69,246	74,721
Insurance proceeds receivable	22,000	22,000
Prepaid expenses and other current assets	8,835	9,762
Property and equipment, net	3,941	4,159
Intangible assets, net	244,093	250,324
Other assets	2,605	1,160
Total assets	$ 447,155	$ 480,782
Accounts payable and accrued liabilities	$ 43,250	$ 54,789
Provision for legal settlement	63,011	64,010
Convertible Notes, net	233,325	225,584
Other noncurrent liabilities	631	612
Total liabilities	340,217	344,995
Total stockholders' equity	106,938	135,787
Total liabilities and stockholders' equity	$ 447,155	$ 480,782

Novelion Therapeutics Inc.

Reconciliation of GAAP to Non-GAAP Financial Information

(unaudited)

	Three Months Ended March 31,	
(In 000s)	**2017**	**2016**
Net loss reconciliation:		
GAAP net loss	$ (30,962)	$ (21,894)
Stock based compensation	1,399	—
Amortization of acquired intangible assets	6,231	—
Amortization of debt discount	7,742	—
Inventory fair value step-up	5,452	—
2016 Aegerion non-GAAP net loss (Net 1)	—	(49,101)
Restructuring charge related to acquisition	1,451	—
Non-GAAP net loss	$ (8,687)	$ (70,995)
GAAP net loss per common share - basic and diluted	$ (1.67)	$ (2.07)
Non-GAAP net loss per common share - basic	$ (0.47)	$ (6.72)
GAAP and Non-GAAP weighted-average common shares outstanding — basic	18,540	10,565
Net revenues reconciliation:		
GAAP net revenues	$ 29,984	$ —
2016 Aegerion revenues (Note 1)	—	35,716

Non-GAAP net revenues	$ 29,984	$ 35,716
Cost of product sales reconciliation:		
GAAP cost of product sales	$ 16,445	$ —
Amortization of acquired intangible assets	(6,231)	—
Inventory fair value step-up	(5,109)	—
Aegerion non-GAAP cost of product sales (Note 1)	—	9,473
Non-GAAP cost of product sales	$ 5,105	$ 9,473
Selling, general and administrative reconciliation:		
GAAP selling, general and administrative	$ 24,451	$ 5,936
Stock based compensation	$ (1,124)	$ —
Inventory fair value step-up	(343)	—
Aegerion non-GAAP SG&A (Note 1)	$ —	$ 36,112
Non-GAAP selling, general and administrative	$ 22,984	$ 42,048
Research and development reconciliation:		
GAAP research and development	$ 9,300	$ 2,990
Stock based compensation	(275)	—
Aegerion non-GAAP R&D (Note 1)	—	9,533
Non-GAAP research and development	$ 9,025	$ 12,523

Note 1 - Includes financial information from pre-merger Aegerion for the three months ended March 31, 2016, excluding stock based compensation, amortization of acquired intangible assets, amortization of debt discount and deferred financing fees, inventory fair value step-up, and restructuring expense.

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