

## **WILSON THERAPEUTICS AB (PUBL)**

## YEAR-END REPORT 2016

## October 1 - December 31, 2016

- Net sales amounted to SEK 0.0 M (0.0)
- Loss for the period was SEK 34.1 M (loss: 21.3)
- Loss per share, before and after dilution, totaled SEK 1.33 (loss: 16.84)
- At December 31, cash and cash equivalents amounted to SEK 386.6 M (31.4)

## January 1 - December 31, 2016

- Net sales amounted to SEK 0.0 M (0.0)
- Loss for the period was SEK 115.2 M (loss: 70.5)
- Loss per share, before and after dilution, totaled SEK 6.84 (loss: 55.53)
- The board of directors proposes that no dividend be paid for 2016

## Significant events during 2016

- A new share issue raised SEK 39.9 M after issue costs
- Resolution regarding a 1:10 share split implemented
- Data from the ongoing Phase 2 trial of WTX101 presented at major medical conferences
- Wilson Therapeutics listed in the Mid Cap segment on Nasdaq Stockholm, raising SEK 402.7 M after issue costs
- Management team expanded
- Clinical Phase 2 study successfully completed

## Significant events after the end of the report period

Extraordinary general meeting was held in January 2017

"We ended 2016 with great news, reporting the successful completion of our Phase 2 study. We are very encouraged by the data and are now focusing on finalizing the design of the Phase 3 program, which we expect to start in the second half of 2017."

Jonas Hansson, CEO, Wilson Therapeutics.

## Financial overview of the Group

Amounts in SEK 000s	Oct-Dec		Jan-Dec	
	2016	2015	2016	2015
Net sales	-	-	-	-
Operating loss	-32,854	-21,287	-113,859	-70,283
Loss for the period	-34,124	-21,366	-115,175	-70,507
Loss per share, before/after dilution (SEK)1)	-1.33	-16.84	-6.84	-55.53
Equity at the end of the period	423,458	84,562	423,458	84,562
Cash flow from operating activities	-28,716	-18,418	-86,148	-61,495
Cash and cash equivalents at the end of the period	386,568	31,404	386,568	31,404

<sup>1)</sup> Adjusted for share split 1:10 resolved in April 2016.



## **CEO STATEMENT**

## Clinical Phase 2 trial successfully completed

We ended 2016 with great news, reporting the successful completion of our Phase 2 study. We are very encouraged by the data, showing that once-daily dosing of WTX101 results in a rapid and significant improvement of free copper levels, which is the underlying cause of Wilson Disease. Furthermore, the patients' symptoms were ameliorated with significant improvements in neurological status and patient reported disability. Also, WTX101 was generally well-tolerated and we did not observe any case of initial drug-induced neurological worsening, a serious and well-described side effect of the existing therapies for Wilson Disease.

The trial was an open label, non-comparator study, which means that all 28 subjects in the study received WTX101 as monotherapy. The study was conducted at 11 centers in the US and EU, with the aim of learning more about the dosing of WTX101, as well as the proposed clinical endpoints for the upcoming pivotal Phase 3 program.

Strengthened by these positive results, we are now focusing on finalizing the design of the Phase 3 program together with the regulatory authorities. During the last few months, we have met with both the FDA and the EMA to discuss our development program. These discussions have so far been very constructive and we plan to conclude these discussions during the first half of 2017 and expect to start the Phase 3 trial in the second half of the year.

#### Wilson Therapeutics completed IPO

On May 12, Wilson Therapeutics completed its Initial Public Offering (IPO) and was listed in the Mid Cap segment on Nasdaq Stockholm. The IPO received strong interest both in Sweden and internationally and the offering was well oversubscribed. The existing main shareholders all subscribed for new shares in the offering alongside several other well-renowned institutional long term investors. There was also a significant interest from retail investors; in all approximately 3,000 new shareholders participated in the offering.

## Fully financed to take WTX101 through clinical development

Operating costs amounted to SEK 113.9 M (70.3) in 2016. The increase was due mainly to higher clinical costs related to the ongoing trial, costs related to the IPO as well as higher costs for our stock option program that have no cash effect. In March, a new share issue raised SEK 40 M after issue expenses from the company's existing shareholders. We raised an additional SEK 403 M after issue expenses in the IPO. This means that we have secured long term financing that is expected to be sufficient to take WTX101 all the way through clinical development in the US and EU, provided the feedback from regulatory authorities continues to support the current development plan.

#### Focus on Phase 3

Our primary objective for 2017 will be to prepare for and initiate the pivotal Phase 3 trial. We are well set up for this with positive Phase 2 data, strong finances and, thanks to several highly successful 2016 hires, an experienced international team.

There have not been any new drugs developed for Wilson Disease in several decades, and significant unmet medical needs still exist. As WTX101 has shown potential to address these unmet needs, the physicians, as well as the patients and their families, are very supportive of our work on WTX101. This is a great source of inspiration for all of us at the company, and when

## YEAR-END REPORT 2016



we initiate the Phase 3 trial in 2017, we will take another big step towards reaching our overall goal of improving the lives of patients with Wilson Disease.

Stockholm February 23, 2017



Jonas Hansson CEO, Wilson Therapeutics AB (publ)



## **PROJECT OVERVIEW**

## **Pipeline**

Product	Indication	Pre-clinical	Phase 1	Phase 2	Phase 3	Reg.
WTX101	Wilson Disease					
	Familial ALS					

#### Wilson Disease

Wilson Disease is a rare genetic disorder of impaired copper metabolism that causes serious copper poisoning. The genetic defect severely affects the body's ability to regulate copper balance, resulting in life-threatening damage to the liver, the brain and further organs if left untreated. Wilson Disease affects approximately one in every 30,000 people worldwide, corresponding to a prevalence of approximately 10,000 patients in the US and 15,000 patients in the EU.

The treatment goal in Wilson Disease is to reduce and maintain free copper at normal levels and as a result to improve the patients' symptoms. The drugs available today for the treatment of Wilson Disease are penicillamine and trientine (so called copper chelators), and zinc. Copper chelators bind and reduce the body's copper levels by increasing urinary copper excretion. Zinc reduces the dietary uptake copper in the gut.

These treatments were introduced in the 1950's and 60's and are all associated with significant shortcomings. The process of reducing copper with the copper chelators is relatively slow and the improvement of clinical symptoms can take years. They are non-specific to copper and also bind to other metals like iron, zinc and calcium. The copper chelators also have severe side effects. One of the most serious side effects is that approximately 25 percent of the patients suffering from neurological symptoms experience a drug-induced worsening of neurological symptoms shortly after initiation of therapy. Of the patients experiencing this worsening, up to 50 percent never recover.

The chelators should be administered up to four times daily on an empty stomach, which often leads to poor treatment compliance. Zinc is not recommended as an initial therapy of symptomatic patients with Wilson Disease because of the slow onset of action. Zinc therapy has been shown to be less efficacious than chelation therapy, and gastrointestinal symptoms such as discomfort or pain are relatively common side effects. Like the chelators, zinc should also be taken multiple (up to five) times per day on an empty stomach.

## **WTX101**

WTX101 (bis-choline tetrathiomolybdate; Decuprate®) is a first in class copper modulating agent with a unique mechanism of action, under investigation as a novel therapy for Wilson Disease. WTX101, unlike current treatments for Wilson Disease, exhibits a specific copper modulating



activity and acts in the liver where it removes copper from the overloaded copper buffer. WTX101 also rapidly neutralizes toxic free copper in tissue and blood by forming complexes with excess copper and albumin. The excess copper is excreted via the bile, the body's natural route for excess copper elimination.

The active ingredient of WTX101, tetrathiomolybdate, has been tested in several clinical studies in Wilson Disease patients and the data from these studies, as well as data from the Company's WTX101-201 study, suggest that WTX101 can rapidly lower and control toxic free copper levels and improve clinical symptoms in these patients. The data also suggest that WTX101 is generally well-tolerated and may have potential for a reduced risk of neurological worsening after initiation of therapy. WTX101 is expected to have a once-daily dosing regimen which may potentially translate into improved compliance in Wilson Disease patients, leading to fewer treatment failures and ultimately improved outcomes as a result. WTX101 has received orphan drug designation for the treatment of Wilson Disease in the US and EU.

#### **Project status**

In November 2016, the Phase 2 study WTX101-201 was concluded. The study was a 24-week open-label Phase 2 study evaluating the efficacy and safety of WTX101 monotherapy in 28 newly-diagnosed patients with Wilson Disease, aged 18 years and older, who had received either no prior treatment for Wilson Disease or a standard of care agent for up to two years. The study was conducted at 11 sites in the U.S. and Europe. All patients who successfully completed the 24-week study period elected to stay on WTX101 in an extension phase of the study.

On December 5, topline study data were reported. In the Intention To Treat population, WTX101 monotherapy reduced mean serum free copper by 77% at week 24 compared to baseline. WTX101 treatment also resulted in significant improvements in neurological status and disability measured as a change from baseline in Unified Wilson Disease Rating Scale (UWDRS) Part 3 and Part 2 respectively. In addition, liver status, as measured by the Modified Nazer Score, was stabilized or improved in the majority of patients.

Treatment with WTX101 was generally well tolerated with most reported adverse events being mild (grade 1) to moderate (grade 2). Reversible liver test elevations were observed in 39% of patients and these elevations were generally mild to moderate, asymptomatic and normalized with dose adjustments. No initial drug-induced neurological worsening was observed upon treatment initiation with WTX101.

Detailed data from the WTX101-201 study will be presented at upcoming medical meetings and a pivotal Phase 3 trial is expected to be initiated in the second half of 2017.

#### **ALS**

Amyotrophic lateral sclerosis (ALS) is a neurodegenerative disease, in which the nerve cells controlling the body's muscles gradually atrophy, leading to general paralysis and respiratory failure. There is one approved drug on the market that prolongs survival by a few months, but there is no effective treatment to control the disease. Median survival for an ALS patient is three to five years. About 1.5 to 2.5 per 100,000 people are diagnosed with ALS every year, and about 30,000 people suffer from the disease in the EU and the US. About 10 percent of patients have a family history of ALS (familial ALS). Of the patients with familial ALS, about 20 percent have a mutation in the gene coding for a copper dependent enzyme known as superoxide dismutase 1 (SOD1).

## YEAR-END REPORT 2016



The active ingredient of WTX101, tetrathiomolybdate, has been tested in mice that are genetically modified with a mutant form of human SOD1 and develop ALS. These studies show that tetrathiomolybdate can both delay the onset of disease as well as reduce the symptoms after disease onset in this mouse model.

## **Project status**

Wilson Therapeutics is exploring the possibility of developing WTX101 for the treatment of patients with SOD1-mutated ALS.



## SIGNIFICANT EVENTS

## **Events during 2016**

## New share issue executed

In March, a new share issue raised SEK 40.0 M from the company's principal shareholders, before issue expenses of SEK 0.1 M. The issue comprised 1,510,840 Class B preference shares, adjusted for a 1:10 share split resolved in April 2016. The issue was conditional on the recruitment of patients for the company's ongoing clinical Phase 2 trial of WX101 progressing according to plan, and the issue was executed when the recruitment target was achieved.

## Resolution regarding a 1:10 share split implemented

The Annual General Meeting on April 4 resolved to implement a 1:10 share split. After the share split, the total number of shares was 16,830,100, comprising 1,450,000 ordinary shares, 3,018,200 Class A preference shares and 12,361,900 Class B preference shares. The share split also impacted the company's employee stock option program and increased the number of options outstanding 1:10.

# Data from the ongoing Phase 2 trial of WTX101 were presented at major European medical conferences

In April, data from the company's ongoing Phase 2 trial of WTX101 were presented at the International Liver Congress<sup>TM</sup> 2016 in Barcelona. The presentation was made by Prof. Karl Heinz Weiss from the University of Heidelberg. Additional data from the study were presented at the 2<sup>nd</sup> Congress of the European Academy of Neurology in Copenhagen in May, and at the 20th International Congress of Parkinson's Disease and Movement Disorders in Berlin in June. These presentations were made by Prof. Anna Czlonkowska from the Medical University of Warsaw. In November, additional data from the clinical trial were presented at the Liver Meeting<sup>®</sup>, organized by the American Association for the Study of Liver Diseases (AASLD), in Boston, Massachusetts. The presentation of the data was provided by Michael Schilsky (MD, FAASLD), Associate Professor at Yale Medical Center and Director, Center of Excellence for Wilson Disease.

## Wilson Therapeutics was listed on Nasdaq Stockholm in the Mid Cap segment

On May 12, Wilson Therapeutics completed its IPO and was listed on Nasdaq Stockholm in the Mid Cap segment. In connection with the listing, all existing preference shares were converted into common shares and 8,890,148 new common shares were issued. The introduction price was set at SEK 49 per share and the IPO raised SEK 435.6 M before issue expenses of SEK 32.9 M. The number of shares outstanding after the IPO amounts to 25,720,248.

## Management team expanded

In June, Rick Lilley was appointed as Chief Regulatory Officer, and in August, Vincent Metzler was appointed as Vice President Commercial Planning & Launch Strategy. Both report to CEO Jonas Hansson and are members of the company's management team.

## Phase 2 study of WTX101 successfully concluded

In December, topline data from the clinical Phase 2 trial with WTX101 were reported. The primary endpoint was met and the mean serum free copper levels were significantly reduced. WTX101 treatment also resulted in significant improvements in neurological status and disability and, in addition, liver status was stabilized or improved in the majority of patients. Treatment with WTX101 was generally well tolerated with most reported adverse events being mild to moderate.



## Events after the end of the report period

## Extraordinary general meeting held

An extraordinary general meeting was held on January 18, 2017. The meeting resolved to implement a new long term incentive program (LTIP 2016) for certain senior executives and key employees in the Wilson Therapeutics group and to implement a similar performance based long term incentive program (Board LTIP 2016) for certain board members of the company. In order to ensure delivery of shares under these programs, the general meeting further resolved to issue not more than 392,500 warrants. The general meeting also resolved to authorize the board, for the period until the next annual general meeting, to issue new shares, provided however that such issues must not exceed ten percent of the total number of shares outstanding in the company.



## FINANCIAL INFORMATION

#### Sales and earnings for the fourth quarter of 2016

Sales amounted to SEK 0.0 M (0.0) and the operating result deteriorated to a loss of SEK 32.9 M (loss: 21.3). The loss for the fourth quarter was SEK 34.1 M (loss: 21.4). The cost increase was due mainly to higher costs related to the concluded Phase 2 clinical trial and costs for the company's stock option program.

## Sales and earnings for the full year 2016

#### Revenue

Sales amounted to SEK 0.0 M (0.0) during the period. The company is not expected to generate any revenue until its products are launched onto the market.

## Sales and administrative expenses

During the year, sales and administrative expenses rose to SEK 42.2 M (17.4). The increase was mainly due to the cost of the IPO amounting to SEK 7.4 M (0.2), and for the build-up of a finance and a marketing function. Recorded non-cash costs for the company's employee stock option program also increased significantly to SEK 12.4 M (4.6).

## Research and development expenditure

During the period, research and development expenditure increased to SEK 70.9 M (53.0). The increase was mainly due to increased costs for the Phase 2 clinical trial and to increased non-cash costs for the company's employee stock option program.

#### **Earnings**

Loss for the year totaled SEK 115.1 M (loss: 70.5), resulting in a loss per share, before and after dilution, of SEK 6.84 (loss: 55.53).

## Liquidity and financing

In 2016, cash flow from operating activities deteriorated to a negative SEK 86.1 M (neg: 61.5), largely due to increased costs for clinical trials and for the IPO. Cash flow from investing activities was a negative SEK 0.2 M (0.0) due to the payment of a rental deposit and acquisitions of equipment.

Cash flow from financing activities amounted to SEK 442.6. M (69.9), attributable to new share issues. The first new share issue, that was executed in March 2016, comprised 1,510,840 Class B preference shares adjusted for a 1:10 share split resolved in April 2016, and raised SEK 40.0 M before issue costs of SEK 0.1 M. In connection with the IPO in May all existing preference shares were converted into common shares and 8,890,148 new common shares were issued, which raised SEK 435.6 M before issue costs of SEK 32.9 M. In total the new share issues raised SEK 475.6 M (30.0) before issue costs of SEK 33.0 M (0.1).

Cash flow for the year was SEK 356.3 M (8.4). At December 31, 2016, cash and cash equivalents amounted to SEK 386.5 M, compared with SEK 31.4 M at December 31, 2015.



## Share-based incentive programs

The purpose of share-based incentive programs is to promote the company's long-term interests by motivating and rewarding the company's senior executives and other employees.

Wilson Therapeutics currently has one employee stock option program comprising employees, certain board members and certain consultants. Program costs of SEK 19.0 M (7.2) were charged to earnings for the period but have no cash impact. During 2016, 110,000 stock options have been granted and no stock options have been exercised. At December 31, 2016, the number of granted stock options amounted to 1,765,000 (1,655,000), where the number of stock options outstanding in 2015 has been adjusted for share split 1:10 decided in April 2016. The company's other provisions amounting to SEK 11.2 M (3.4) are provisions for social contributions related to the stock option program.

After the end of the report period, in February 2017, a new long term incentive program for certain senior executives and key employees and similar program for certain board members were implemented. Both programs are three-year performance based share programs under which the participants will be granted not more than 392,500 shares in Wilson Therapeutics in total.

#### Investments

Fixed assets mainly consist of intellectual property rights. During 2016 investments in intangible fixed assets amounted to SEK 0.0 M (0.0), and investments in tangible fixed assets to SEK 0.1 M (0.0).

## **Employees**

At December 31, 2016, Wilson Therapeutics had 14 (7) employees. The average number of employees was 10 (6) in 2016.

#### **Parent Company**

Since the operations of the Parent Company are consistent with those of the Group in all material respects, the comments for the Group are also largely relevant for the Parent Company.

## Annual general meeting

The Annual General Meeting will be held on May 17, 2017 at 2.00 pm at Berns (Ljusgården), Bezelii park, Stockholm Sweden. The Annual Report for 2016 will be available at Wilson Therapeutics' office at Kungsgatan 3 in Stockholm and on the company's website at least three weeks prior to the AGM.

## **Proposed dividend**

Since Wilson Therapeutics is currently in an expansive and capital-intensive phase, the Board proposes that the AGM resolve to pay no dividend for 2016.



## CONDENSED CONSOLIDATED STATEMENT OF COMPREHENSIVE INCOME

Amounts in SEK 000s	Oct-Dec		Jan-Dec		
	2016	2015	2016	2015	
Net sales	-	-	-	-	
Gross profit	-	-	-	-	
Sales and administrative expenses	-9,263	-6,403	-42,208	-17,357	
Research and development expenses	-23,149	-14,919	-70,851	-52,961	
Other operating revenue	-	35	20	35	
Other operating expenses	-442	-	-820	-	
Operating loss	-32,854	-21,287	-113,859	-70,283	
Net financial items	-1,243	-10	-1,245	-57	
Loss before tax	-34,097	-21,297	-115,104	-70,340	
Tax	-27	-69	-71	-167	
Loss for the period <sup>1)</sup>	-34,124	-21,366	-115,175	-70,507	
Other comprehensive income					
Items that will be reclassified to profit or loss		_			
Translation difference for the period	83	8	123	44	
Other comprehensive income after tax for	83	8	122	44	
Comprehensive loss for the period <sup>1)</sup>	-34,041	-21,358	-115,052	-70,463	
Loss per share, before/after dilution (SEK) <sup>2)</sup>	-1.33	-16.84	-6.84	-55.53	

<sup>1) 100%</sup> attributable to Parent Company shareholders.

<sup>2)</sup> Adjusted for share split 1:10 resolved in April 2016. Earnings per share calculated as earnings per common share, where the result is adjusted for the right of preference shareholders to receive a dividend for the period. During the period, all preference shares have been converted into common shares in connection with the stock market listing.



## CONDENSED CONSOLIDATED STATEMENT OF FINANCIAL POSITION

Amounts in SEK 000s	DEC 31, 2016	DEC 31, 2015
ASSETS		
Intangible fixed assets	64,632	64,632
Tangible fixed assets	55	13
Financial fixed assets	151	54
Total fixed assets	64,838	64,699
Current receivables	2,480	2,164
Cash and cash equivalents	386,568	31,404
Total current assets	389,048	33,568
TOTAL ASSETS	453,886	98,267
EQUITY AND LIABILITIES		
Equity attributable to shareholders of the parent company	423,458	84,562
Other provisions	11,167	3,447
Total non-current liabilities	11,167	3,447
Accounts payable	8,155	3,119
Other current liabilities	11,106	7,139
Total current liabilities	19,261	10,258
TOTAL EQUITY AND LIABILITIES	453,886	98,267



## CONDENSED CONSOLIDATED STATEMENT OF CHANGES IN EQUITY

Amounts in SEK 000s	Oct-	Dec	Jan-Dec	
	2016	2015	2016	2015
Opening balance	455,354	63,073	84,562	80,125
Comprehensive loss for the period	-34,041	-21,358	-115,052	-70,463
New share issue	-	40,000	475,617	70,000
Costs attributable to new share issue	-	-20	-32,976	-107
Employee stock option program	2,145	2,867	11,307	5,007
Total transactions with owners	2,145	42,847	453,948	74,900
Closing balance	423,458	84,562	423,458	84,562

## CONDENSED CONSOLIDATED STATEMENT OF CASH FLOW

Amounts in SEK 000s	Oct-	Dec	Jan-	Dec
	2016	2015	2016	2015
Operating loss	-32,854	-21,287	-113,859	-70,283
Adjustment for non-cash items1)	3,291	4,019	19,047	7,262
Interest received	2	0	3	0
Interest paid	0	-2	-9	-5
Tax paid	4	-159	-68	-267
Cash flow from operating activities before changes in working capital	-29,557	-17,429	-94,886	-63,293
Cash flow from changes in working capital	841	-989	8,738	1,798
Cash flow from operating activities	-28,716	-18,418	-86,148	-61,495
Cash flow from investing activities	31	-	-160	-
Cash flow from financing activities	-	39,980	442,641	69,893
Cash flow for the period	-28,685	21,562	356,333	8,398
Cash and cash equivalents at the beginning of the period	416,462	9,853	31,404	23,011
Exchange-rate difference in cash and cash equivalents	-1,209	-11	-1,169	-5
Cash and cash equivalents at the end of the period	386,568	31,404	386,568	31,404

<sup>1)</sup> Pertains mainly to costs of employee stock option program including social contributions.



## CONDENSED PARENT COMPANY PROFIT AND LOSS STATEMENT

Amounts in SEK 000s	Oct-Dec		Jan-Dec	
	2016	2015	2016	2015
Net sales	-	-	-	-
Gross profit	-	-	-	-
Sales and administrative expenses	-9,253	-6,385	-42,116	-17,180
Research and development expenditure	-23,773	-15,888	-71,489	-53,576
Other operating revenue	0	35	0	35
Other operating expenses	-442	-	-820	-
Operating loss	-33,468	-22,238	-114,425	-70,721
Profit/loss from financial items	-1,243	-36	-1,245	-82
Loss after financial items	-34,711	-22,274	-115,670	-70,803
Tax	-	-	-	-
Loss for the period	-34,711	-22,274	-115,670	-70,803

## CONDENSED PARENT COMPANY STATEMENT OF COMPREHENSIVE INCOME

Amounts in SEK 000s	Oct-Dec		Jan-	Dec
	2016	2015	2016	2015
Loss for the period	-34,711	-22,274	-115,670	-70,803
Other comprehensive income	-	-	-	-
Comprehensive loss for the period	-34,711	-22,274	-115,670	-70,803



## CONDENSED PARENT COMPANY BALANCE SHEET

Amounts in SEK 000s	DEC 31, 2016	DEC 31, 2015
ASSETS		
Intangible fixed assets	32,360	32,360
Tangible fixed assets	55	13
Financial fixed assets	32,582	32,485
Total fixed assets	64,997	64,858
Current receivables	2,560	2,237
Cash and cash equivalents	385,498	31,063
Total current assets	388,058	33,300
TOTAL ASSETS	453,055	98,158
EQUITY AND LIABILITIES		
Restricted shareholders' equity	2,858	1,702
Unrestricted shareholders' equity	419,071	81,949
Total Equity	421,929	83,651
Other provisions	11,167	3,447
Accounts payable	8,147	3,096
Other current liabilities	11,812	7,964
Total current liabilities	19,959	11,060
TOTAL EQUITY AND LIABILITIES	453,055	98,158



## **NOTES**

#### Note 1 Accounting policies in accordance with IFRS

This interim report for the Group has been prepared in accordance with IAS 34 Interim Financial Reporting and the applicable provisions of the Swedish Annual Accounts Act. The interim report for the Parent Company has been prepared pursuant to Chapter 9 of the Swedish Annual Accounts Act, Interim Financial Reporting. The same accounting policies and measurement criteria have been applied for the Group and the Parent Company as in the 2015 Annual Report. New or revised IFRS requirements introduced in 2016 have not affected Wilson Therapeutics during the period.

New ESMA guidelines for key ratios have taken effect during the period. The group presents the alternative key ratio operating profit/loss as it is deemed useful as a complement to other key ratios to understand the group's financial reports. The group does not present any other alternative key ratios as no such key ratios are deemed necessary to understand the group activities.

## Note 2 Risks and uncertainties in the Group and Parent Company

#### **Operational risks**

Research and drug development up to approved registration is subject to considerable risk and is a capital-intensive process. The majority of all initiated projects will never reach market registration due to the technological risk such as the risk for insufficient efficacy, intolerable side effects or manufacturing problems. If competing pharmaceuticals capture market share or reach the market faster, or if competing research projects achieve better product profile, the future value of the product portfolio may be lower than expected. The operations may also be impacted negatively by regulatory decisions, such as approvals and price changes.

## Financial risk management

Wilson Therapeutics' financial policy governing the management of financial risks has been designed by the Board of Directors and represents a framework of guidelines and rules in the form of risk mandates and limits for financial activities. Wilson Therapeutics is primarily affected by foreign-exchange risk. A considerable portion of the company's costs is denominated in USD and EUR. In accordance with the established financial policy, no currency hedging has been employed during the period. However, a portion of the company's cash, amounting to the expected need for the coming 12-month period, is held in USD. The financial policy is updated at least once annually.

For a more detailed description of risks and uncertainties, refer to Note 18 in the 2015 Annual Report.

#### **Note 3 Financial instruments**

Wilson Therapeutics' financial assets and liabilities comprise cash and cash equivalents, accrued expenses and accounts payable. Therefore, the fair values of all financial instruments are approximately equal to their carrying amounts, since all maturities are short. Wilson Therapeutics has not applied net accounting to any financial assets or liabilities, and has no agreements that permit offsetting.

## Note 4 Cash and cash equivalents

The company's cash and cash equivalents consists of cash deposits at the company's bank.



## Note 5 Change in number of shares

	Oct-	Oct-Dec		Jan-Dec	
	2016	2015	2016	2015	
Total number of shares, opening balance	25,720,248	1,131,935	1,531,926	731,928	
Shares added through share split 1:10	-	-	15,147,090	-	
Shares added through new share issues	-	399,991	9,041,232	799,998	
Total number of shares, closing balance	25,720,248	1,531,926	25,720,248	1,531,926	

#### Note 6 Related-party transactions

During the period a new share issue directed at the major shareholders was executed. No other transactions have taken place between the company and its related parties that could materially affect the company's position and earnings for the period.

## Note 7 Estimates and judgments

Preparation of the interim report requires management to make estimates that affect the reported amounts of assets, liabilities, revenues and expenses. Actual outcomes may deviate from these estimates. The key sources of estimation uncertainty are the same as those outlined in Note 2 of the 2015 Annual Report.

## Review

This year-end report has not been reviewed by the company's auditors.

Stockholm February 23, 2017

Jonas Hansson CEO

The information in the year-end report is information that Wilson Therapeutics is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact person set out above, at 08.00 CET on February 23, 2017.



#### For further information contact:

Jonas Hansson, CEO, Wilson Therapeutics AB Telephone: +46 8 796 00 00 Email: jonas.hansson@wtx.se

Wilson Therapeutics AB (publ) Corp. Reg. No. 556893-0357 Kungsgatan 3 SE-111 43 Stockholm

#### Financial calendar

Interim report for the first quarter 2017 May 17, 2017
Annual General meeting 2017 May 17, 2017
Interim report for the second quarter 2017 August 24, 2017
Interim report for the third quarter 2017 November 23, 2017

## **About Wilson Therapeutics**

Wilson Therapeutics is a biopharmaceutical company, based in Stockholm, Sweden, that develops novel therapies for patients with rare diseases. Wilson Therapeutics' lead product, WTX101, is initially being developed as a novel treatment for Wilson Disease and has been evaluated in a Phase 2 clinical study. Wilson Therapeutics is listed in the Mid Cap segment on Nasdaq Stockholm with the stock ticker WTX.

Visit <u>www.wilsontherapeutics.com</u> for more information.