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## Investigational Drug Telaprevir Promising in Treatment of Hepatitis C

*According to the publication Science Daily, adding the investigational drug telaprevir to standard treatment of hepatitis C infection cures about half of the patients willing to give therapy a second try. Typically the cure rate is about 14 percent among those who were treated with the standard regimen of peginterferon and ribavirin. When telaprevir was used in combination with the older anti-viral treatments, cure rates were noted to be substantial.*

Treatment of hepatitis C is historically challenging as available treatment options are uncomfortable for the patient and sometimes are ineffective. Researchers at Duke Clinical Research Institute found that patients who had not improved with standard HCV treatment significantly improved response rates when telaprevir was added to the standard treatment regimen.

Researchers looked at 117 patients, all of whom received treatment with the combination of pegylated interferon alpha, ribavirin and telaprevir. All patients received 12 weeks of triple combination and then 12 to 36 more weeks of pegylated interferon alfa and ribavirin.

Telaprevir was discontinued after the initial 12 weeks. However, results showed that null responders- patients who did not respond to previous treatment needed 48 weeks of treatment, but 57 percent of null responders were cured with this regimen.

The other groups received 12 weeks of triple combination therapy plus 12 more weeks of peginterferon alfa and ribavirin. Of the patients who were partial responders of the previous therapy, 60 percent were cured with triple combination. For patients who had relapsed with previous therapy, 92 percent were cured with the triple combination.

Should these findings continue in a larger phase III study, it could provide a meaningful treatment option for clinicians when dealing with patients who have failed an initial standard of care treatment regimen.

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## Oral Therapy on the Horizon for Treatment of Gaucher Disease

*Gaucher disease is a rare enzyme deficiency disorder in which a person lacks the enzyme glucocerebrosidase. The lack of glucocerebrosidase enzyme causes harmful substances to build up in the liver, spleen, bones and bone marrow.*

These substances prevent cells and organs from working properly. Symptoms vary depending upon the type of disease but it can include bone pain and fractures, cognitive impairment, bruising, enlarged spleen and liver, fatigue, heart valve problems, lung disease and seizures. Gaucher disease affects an estimated 1 in 50,000 to 1 in 100,000 people in the general U.S. population.

Gaucher is an autosomal recessive disease. This means that the mother and father must both pass one abnormal copy of the gene to the child to develop the disease. There are three main types of Gaucher disease:

- Type 1 disease is the most common. It involves bone disease, anemia, an enlarged spleen and thrombocytopenia. Type 1 affects both children and adults and is most common in the Ashkenazi Jewish population.
- Type 2 disease usually begins in infancy with severe neurologic involvement. This form can lead to rapid, early death.
- Type 3 disease may cause liver, spleen and brain problems. Patients may live into adulthood.

How well a person does depends upon the subtype of the disease. The infantile form of Gaucher disease may lead to early death. Most affected children die before age 5. Adults with the type 1 form of the disease can expect normal life expectancy with enzyme replacement therapy.

The primary treatment option is Cerezyme which is administered intravenously approximately every two weeks for life. Depending upon the weight of the patient and severity of the disease the cost of the drug itself is upwards of \$150,000 annually. Currently this is the only treatment option for the disease.

However, in a study published in May in *Blood*, the journal of the American Society of Hematology, researchers present positive results of a Phase II clinical trial of eliglustat tartrate, an oral therapy in development to treat Gaucher disease type 1. As a substrate reduction therapy, the drug decreases the body's production of fatty substances so they do not accumulate in the cells. Dosages for the Phase II trial were 50mg to 100 mg administered twice daily.

The study endpoint (improvement in at least two of the three main efficacy parameters: spleen volume, hemoglobin level and platelet count) was met by three-fourths (77%) of all patients and nearly all (91%) of the 22 patients who completed the full 52 week test. The greatest overall improvements were seen in hemoglobin level and spleen volume. Significant improvements were also seen across many disease symptoms, including hemoglobin level, platelet count, spleen volume, liver volume, and lumbar spine mineral den-

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### Oral Therapy on the Horizon for Treatment of Gaucher Disease, continued

sity. Disease symptoms seemed to respond rapidly. This may have resulted in the drug's small molecular size, which allows it to diffuse quickly within affected cells.

A larger Phase III trial is proceeding to study untreated patients and patients who previously stabilized using Cerezyme. Researchers continue to strive to develop new options with improved efficacy and safety and provide easier alternatives for both the patient and physician.

## New Drug Indications in the Pipeline

Drug Name	Current Indication	Investigational Indication	Route of Administration	Comments
Xolair	Treatment of adults and children 12 years of age and older with moderate to severe asthma	Treatment of children 6 years of age and above with moderate to severe asthma	SC injection	FDA recommended against approval in November 2009.
Cinryze	Routine prophylaxis against angioedema attacks in patients with hereditary angioedema	Treatment of acute angioedema attacks in patients with hereditary angioedema	IV infusion	FDA has requested additional clinical study
H.P. Acthar Gel	Multiple indications	Treatment of infantile spasms	IM or SC injection	FDA response to supplemental new drug application expected June 2010
Zavesca	Treatment of adult patients with mild to moderate type 1 Gaucher	Treatment of progressive neurological manifestations of Niemann-Pick type C disease	Oral	Orphan drug designation
Campath	Treatment of B-cell chronic lymphocytic leukemia	Treatment of relapsing-remitting MS	IV infusion	Supplemental biologics license application expected in 2011
Rituxan	Treatment of non-Hodgkins lymphoma and rheumatoid arthritis	In combination with standard chemo for treatment of chronic lymphocytic leukemia	IV infusion	
Clolar	Treatment of patients age 1 to 21 years with relapsed or refractory acute myloid leukemia	Treatment of adult patients with acute myloid leukemia	IV infusion	Orphan drug designation
Tasigna	Treatment of chronic and accelerated phase Philadelphia chromosome positive chronic myloid leukemia in adults resistant or intolerant to Gleevec	First-line treatment of chronic myloid leukemia and treatment of gastrointestinal stromal tumor in patients who have failed on Gleevec or Sutent	Oral	FDA response to supplemental new drug application expected October 2010

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### New Drug Indications in the Pipeline, continued

Drug Name	Current Indication	Investigational Indication	Route of Administration	Comments
Tarceva	Treatment of advanced non-small cell lung cancer after failure of at least one prior chemo regimen. First-line treatment of advanced pancreatic cancer in combination with Gemzar	First-line maintenance therapy in patients with non-small cell lung cancer.	Oral	
Sutent	Treatment of gastrointestinal stromal tumor and advanced renal cell carcinoma	Treatment of breast cancer, non-small cell lung cancer, hepatocellular cancer, hormone refractory prostate cancer and pancreatic cancer	SC injection	Supplemental biologics license application filed January 2010
Peg-Intron	Treatment of Hepatitis C	Adjuvant treatment of stage III melanoma	SC injection	
Tracleer	Treatment of pulmonary hypertension	Treatment of pulmonary fibrosis	Oral	Phase III results expected in 2010
Certican	Treatment of advanced renal cell carcinoma in patients who have failed Sutent or Nexavar	Prevention of kidney transplant rejection in combination with Neoral	Oral	Marketed under brand name Afinitor for RCC indication

### LDI Specialty Drug Pipeline Report 2nd & 3rd Quarter 2010

Estimated Approval	Brand	Generic	Indications	Place In Therapy	Dosage Form
1Q10*	Actemra	tocilizumab	Rheumatoid Arthritis (RA)		IV
1Q10*	Ampyra	fampridine SR	Multiple Sclerosis (MS)	First in class. Add on therapy to traditional MS agents.	Oral
1Q10*	Carbaglu	carglumic acid	Hyperammonaemia due to N-acetylglutamate synthase deficiency (NAGS deficiency)	Adjunctive therapy for treatment and also used for maintenance.	Oral
1Q10*	Cayston	aztreonam lysine	Cystic Fibrosis (CF)	For use in CF patients with confirmed Pseudomonas aeruginosa.	Inhalation
1Q10*	Menveo	meningococcal vaccine (MenACWY-CRM)	Meningitis	Vaccine	IM

Estimated Approval	Brand	Generic	Indications	Place In Therapy	Dosage Form
1Q10*	Prevnar-13	13-valent pneumococcal conjugate vaccine (PCV13 )	Protection against pneumococcal disease (PD) and otitis media	Vaccine. Replacement of Prevnar. Protects against 13 types of Strep. pneumoniae bacteria compared to Prevnar which protected against 7 strains of the same bacteria.	Injection
1Q10*	Vpriv	velaglucerase alfa (GA-GCB)	Gaucher's disease	Alternative to other enzyme replacement therapies.	IV
1Q10*	Xiaflex	collagenase clostridium histolyticum	Dupuytren's contracture	First in class. Alternative to surgery.	Injection
2Q10	Gilenia	fingolimod (FTY720)	Multiple Sclerosis (MS)	First oral therapy for treatment of Multiple Sclerosis.	Oral
2Q10	N/A	AAT-IV (alpha-1 antitrypsin)	Alpha-1 antitrypsin deficiency	Alternative to other alpha-1 antitrypsin deficiency agents.	IV (liquid, ready to use)
2Q10*	Provenge	sipuleucel-T	Hormone refractory prostate cancer (HRPC)	First in class. Used after failure of other therapies.	IV
3Q10	Egrifta	tesamorelin (TH9507)	HIV-associated lipodystrophy	First drug approved for this indication.	SC
3Q10		motavizumab	RSV	Alternative therapy.	IM
3Q10	Monovisc	hyaluronic acid (HA)	Osteoarthritis of the knee	Alternative therapy.	Intra-articular
3Q10	Esbriet	pirfenidone	Idiopathic pulmonary fibrosis (IPF)	First in class. Orphan drug status.	oral
3Q10	N/A	Adenovirus Types 4 and 7 Live Oral Vaccines	Adenovirus	Vaccine	Oral
3Q10	Neutroval	XM02	Severe neutropenia, febrile neutropenia	Would be first bio-similar drug approval in the United States. Will provide competition to Neupogen.	SC
3Q10	Prolia	denosumab	Osteoporosis (postmenopausal, undergoing hormone ablation for either prostate or breast cancer), bone metastases	Alternative therapy.	SC

\* Recent FDA approval.

# Specialty drug listing

## Acromegaly

OCTREOTIDE ACETATE  
SANDOSTATIN LAR DEPOT  
SANDOSTATIN  
SOMATULINE DEPOT  
SOMAVERT\*

## AIDS

FUZEON  
NEBUPENT  
PENTAMIDINE  
SEROSTIM  
VISTIDE  
ZIDUVUDINE

## Anemia

ARANESP  
EPOGEN  
PROCRIT

## Anticoagulants

ARIXTRA  
FRAGMIN  
INNOHEP  
LOVENOX

## Asthma

XOLAIR\*

## Cancer

ABRAXANE  
ACTIMMUNE  
ADRIAMYCIN  
ADRUCIL  
AFINITOR  
ALIMTA  
ALKERAN  
AVASTIN  
BICNU  
BLENOXANE  
BLEOMYCIN SULFATE  
BUSULFEX  
CAMPATH  
CAMPTOSAR  
CARBOPLATIN  
CASODEX  
CISPLATIN  
CLADRIBINE  
CYCLOPHOSPHAMIDE  
CYTARABINE  
CYTOXAN  
DACARBAZINE  
DACOGEN  
DAUNOXOME  
DOXIL  
DOXORUBICIN HCL  
ELIGARD  
ELLENC  
ELOXATIN  
ELSPAR  
EMCYT  
EPIRUBICIN HCL  
ERBITUX  
ETOPOPHOS  
ETOPOSIDE  
FASLODEX  
FLOXURIDINE  
FLUDARA  
FLUDARABINE PHOSPHATE  
FLUOROURACIL  
FLUTAMIDE

FUDR  
GEMZAR  
GLEEVEC  
HERCEPTIN  
HYCAMTIN  
IFEX  
IFOSFAMIDE  
INTRON-A  
IRESSA\*  
IRINOTECAN  
IXEMPRA  
LEUPROLIDE ACETATE  
LEUSTATIN  
LUPRON DEPOT  
LYSODREN  
MERCAPTOPURINE  
MITOXANTRONE HCL  
MUSTARGEN  
MYLERAN  
MYLOTARG  
NAVELBINE  
NEXAVAR\*  
NIPENT  
NOVANTRONE  
ONCASPAR  
ONTAK  
ONXOL  
PACLITAXEL  
PARAPLATIN  
PENTOSTATIN  
PLATINOL AQ  
PROLEUKIN  
PURINETHOL  
REVLIMID\*  
RITUXAN  
SANDOSTATIN LAR DEPOT  
SPRYCEL  
SUTENT  
TABLOID  
TARCEVA  
TARGRETIN  
TAXOL  
TAXOTERE  
TEMODAR  
THALOMID  
THYROGEN  
TOPOSAR  
TORISEL  
TRELSTAR DEPOT  
TRELSTAR LAR  
TRETINOIN  
TYKERB\*  
VANTAS\*  
VESANOID  
VIDAZA  
VINBLASTINE SULFATE  
VINCASAR PFS  
VINCRISTINE SULFATE  
VINORELBINE TARTRATE  
VOTRIENT  
XELODA  
ZANOSAR  
ZOLADEX  
ZOLINZA  
ZOMETA

## Cancer Adjunct

ALOXI  
ANZEMET  
AREDIA  
ETHYOL

GRANISETRON HCL  
KYTRIL  
LEUCOVORIN CALCIUM  
LEUKINE  
MESNA  
MESNEX  
NEULASTA  
NEUMEGA  
NEUPOGEN  
ONDANSETRON HCL  
PAMIDRONATE DISODIUM  
ZOFRAN

## Cervical Dystonia

BOTOX  
MYOBLOC

## Crohn's Disease

CIMZIA\*  
HUMIRA  
REMICADE  
TYSABRI  
PULMOZYME  
TOBI

## Fabry's Disease

FABRAZYME

## Gaucher's Disease

CEREDASE  
CEREZYME  
VPRIV  
ZAVESCA\*

## Growth Hormone

GENOTROPIN  
HUMATROPE  
NORDITROPIN  
NUTROPIN  
NUTROPIN AQ  
OMNITROPE  
SAIZEN  
TEV-TROPIN

## Hemophilia

ADVATE  
ALPHANATE (VWF)  
ALPHANINE SD  
BEBULIN VH  
BENEFIX  
DDAVP  
FEIBA VH IMMUNO  
HELIXATE FS  
HEMOFIL M  
HUMATE-P  
KOATE-DVI  
KOGENATE FS  
MONARC-M  
MONOCLATE-P  
MONONINE  
NOVOSEVEN  
PROFILNINE SD  
RECOMBINATE  
REFACTO  
STIMATE

## Hepatitis B

BARACLUDGE  
EPIVIR HBV  
HEPAGAM B

HEPSERA  
HYPERHEP B S/D  
NABI-HB

## Hepatitis C

COPEGUS  
INFERGEN  
INTRON-A  
PEGASYS  
PEG-INTRON  
REBETOL  
RIBAPAK  
RIBAVIRIN  
ROFERON-A

## Hunter Syndrome

ELAPRASE\*

## Immunodeficiency

CARIMUNE NF  
FLEBOGAMMA  
GAMMAGARD S/D  
GAMUNEX  
IMMUNE GLOBULIN  
OCTAGAM  
PANGLOBULIN  
POLYGAM S/D  
VIVAGLOBIN

## Infertility

BRAVELLE  
CETROTIDE  
CHORIONIC GONADOTROPIN  
DELESTROGEN  
ESTRADIOL VALERATE  
FOLLISTIM AQ  
GANIRELIX ACETATE  
GONAL-F  
LUVERIS  
MENOPUR  
NOVAREL  
OVIDREL  
PREGNYL  
PROCHIEVE  
PROGESTERONE  
REPRONEX

## Miscellaneous

ALFERON N  
APOKYN\*  
ARALAST\*  
ARCALYST\*  
CARBAGLU  
CINRYZE\*  
CYTOVENE  
EXJADE\*  
H.P. ACTHAR GEL\*  
INCRELEX  
KUVAN\*  
NAGLAZYME\*  
PROLASTIN\*  
SABRIL\*  
SOLIRIS  
VIVITROL  
XIAFLEX\*  
XYREM\*  
ZEMAIRA\*  
ZORBITIVE\*

## Multiple Sclerosis

AMPYRA  
AVONEX  
BETASERON  
COPAXONE  
REBIF  
TYSABRI\*

## Osteoarthritis

EUFLEXXA  
HYALGAN  
ORTHOVISC  
SUPARTZ  
SYNVISIC

## Osteoporosis

FORTEO  
MIACALCIN  
RECLAST

## Psoriasis

AMEVIVE  
ENBREL  
HUMIRA  
REMICADE  
STELARA

## Pulmonary Arterial Hypertension (PAH)

FLOLAN\*  
LETAIRIS  
REMODULIN\*  
REVATIO  
TRACLEER\*  
VENTAVIS\*

## Rh Hemolytic Disease

MICROHOGAM  
RHOGAM  
RHOPHYLAC  
WINRHO SDF

## Rheumatoid Arthritis

ACTEMRA  
CIMZIA\*  
ENBREL  
HUMIRA  
REMICADE  
KINERET  
ORENCIA  
RITUXAN  
SIMPONI

## RSV Prevention

SYNAGIS

\* Indicates drug is on Limited Distribution