

Ukraine

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Denmark LNAA STUDY

1 tablet/kg/day

Blood Samples	PKU 20	PKU 39	PKU 93	PKU 105	PKU 128	Avg
1	1436	1681	1697	1597	1627	1608
02	1262	1691	1591	1480	1602	1525
04	1164	1643	1526	1414	1407	1431
1 week	184	-58	220	184	268	160

Denmark LNAA STUDY

2 tablets/kg/day

Blood Samples	PKU 20	PKU 39	PKU 93	PKU 105	PKU 128	Avg
08	1252	1739	1477	1413	1359	1448
09	1146	1537	1370	1233	1373	1332
11	1119	1556	1389	1179	1313	1311
15	1199	1650	1349	1222	1335	1351
2 week	237	31	348	375	292	257

Russia LNAA STUDY

Time	Phe		Tyr	
	$\mu\text{mol/l}$	mg/dl	$\mu\text{mol/l}$	mg/dl
KA				
0'	718.8	11.98	53.9	0.98
3 days	668.4	11.14	91.3	1.66
3 days	523.2	8.72	103.4	1.88
3 days	376.2	6.27	108.3	1.97

Russia LNAA STUDY

Time

Phe

Tyr

KN

μmol/l

mg/dl

μmol/l

mg/dl

0'

707.4

11.79

42.9

0.78

3 days

607.2

10.12

126.5

2.30

3 days

572.4

9.54

159.5

2.91

3 days

585.6

9.76

83.6

1.52

Russia LNAA Study

Time

Phe

Tyr

KH

$\mu\text{mol/l}$

mg/dl

$\mu\text{mol/l}$

mg/dl

0'

635.4

10.59

33.0

0.60

3 days

554.4

9.24

242.0

4.40

3 days

322.2

5.37

94.6

1.72

3 days

136.2

2.27

110.0

2.00

3 days

102.6

1.71

94.0

1.71

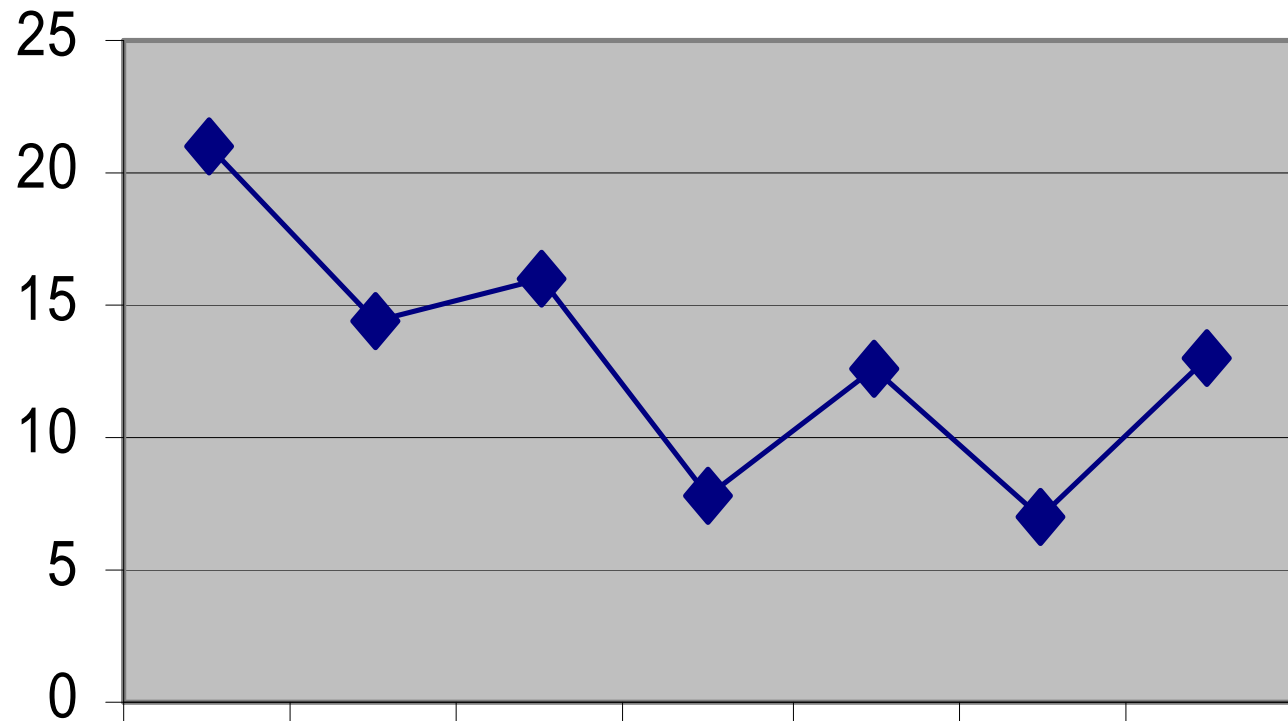
USA LNAA STUDY

Time	Phe		Tyr	
	$\mu\text{mol/l}$	mg/dl	$\mu\text{mol/l}$	mg/dl
GDL				
0'	1290.6	21.51	69.8	1.27
2 days	1198.2	19.97	73.7	1.34
4 days	115.8	1.93	140.25	2.55
KM				
0'	1540.2	25.67	30.8	0.56
8 days	883.8	14.37	53.8	0.98
0'	1978.2	32.97	68.7	1.25
2 days	1608.6	26.81	207.35	3.77

USA LNAA STUDY

Time	Phe		Try	
	$\mu\text{mol/l}$	mg/dl	$\mu\text{mol/l}$	mg/dl
ES				
0'	1375.8	22.93	31.9	0.58
4-7 days	767.4	12.79	121.5	2.12
RC				
0'	965.4	16.09	58.8	1.07
2 days	828.6	13.81	156.2	2.84

Response of Blood Phe to LNAA Ukraine



◆ 21 yo girl	21	14.5	16	7.9	12.7	7.1	13
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µmol/l
426 780

1260

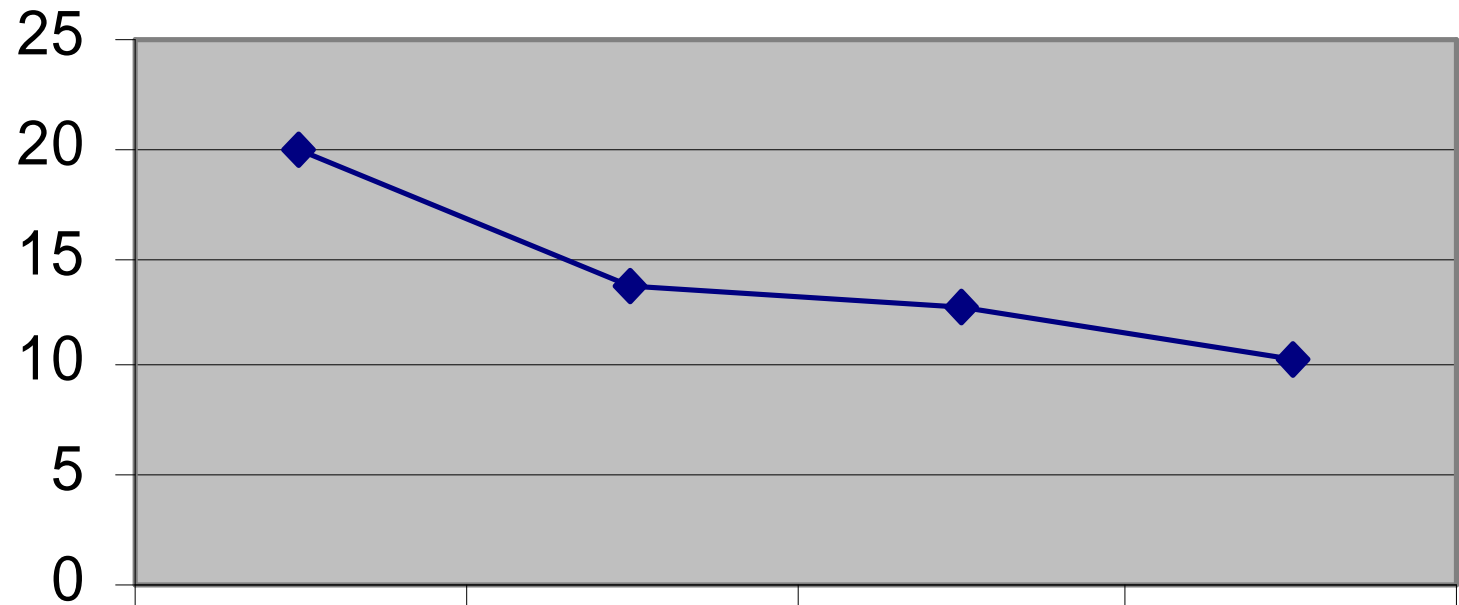
870

960

474

762

Response on Phe on LNAA Ukraine



◆ 12 yo boy	20	13.8	12.75	10.4
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μmol/l
624

1200

828

765

US Blood Phe and Tyr NeoPhe Patient K 1 Week μmol/L (mg)

Control		NeoPhe	
phe	tyr	phe	tyr
		μmol/L (mg)	
1978.1(32.97)	1.25	1356.0 (22.6)	5.0
1139.6 (25.66)	0.62	1308 (21.8)	4.1
1456.2 (24.27)	0.62	1146 (19.1)	3.82

24% reduction

US Blood Phe and Tyr
NeoPhe Patient G 1 Week
 $\mu\text{mol/L}$ (mg)

Control		NeoPhe	
phe	tyr	phe	tyr
mg/dl		mg/dl	
1560 (26.0)	0.92	953 (15.89)	4.35
1764 (29.4)	1.9	505 (8.43)	3.32

56% reduction

NeoPhe 0.5 g/kg in PKU Subjects

- 13 subjects
- Mean age 26.6 years
- 7 males, 6 females
- Mean decrease in blood Phe after one week 243 $\mu\text{mol/L}$
- Average decrease in blood Phe 22 %.

NeoPhe 1.0 g/kg in PKU Subjects

- 7 subjects
- Mean age 25.2 years
- 5 males, 2 females
- Mean decrease in blood Phe after one week 377 $\mu\text{mol/L}$
- Average decrease in blood Phe 25 %.

Figure 1. Blood Phe Response to 0.5g/kg NeoPhe in Patients with PKU

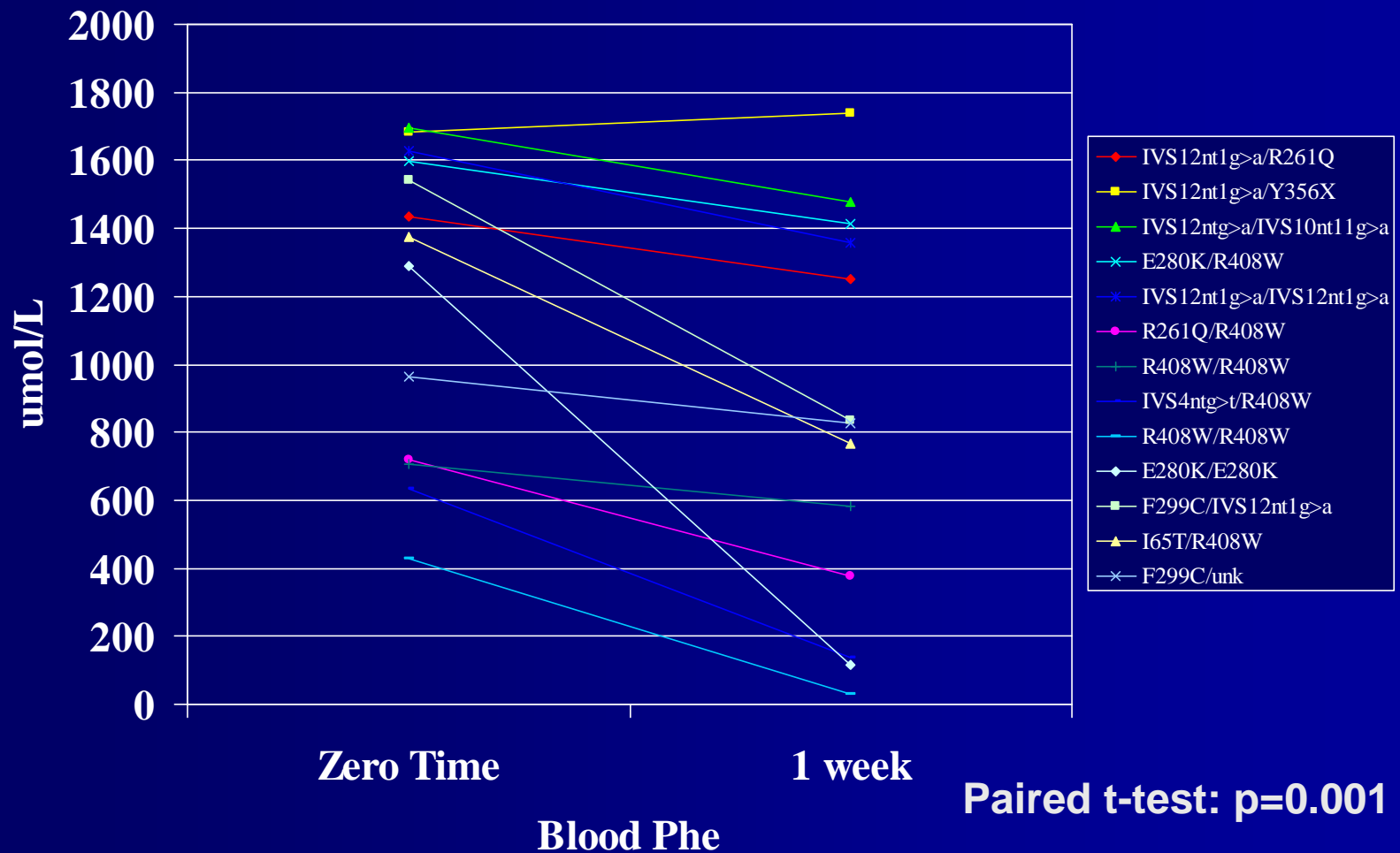
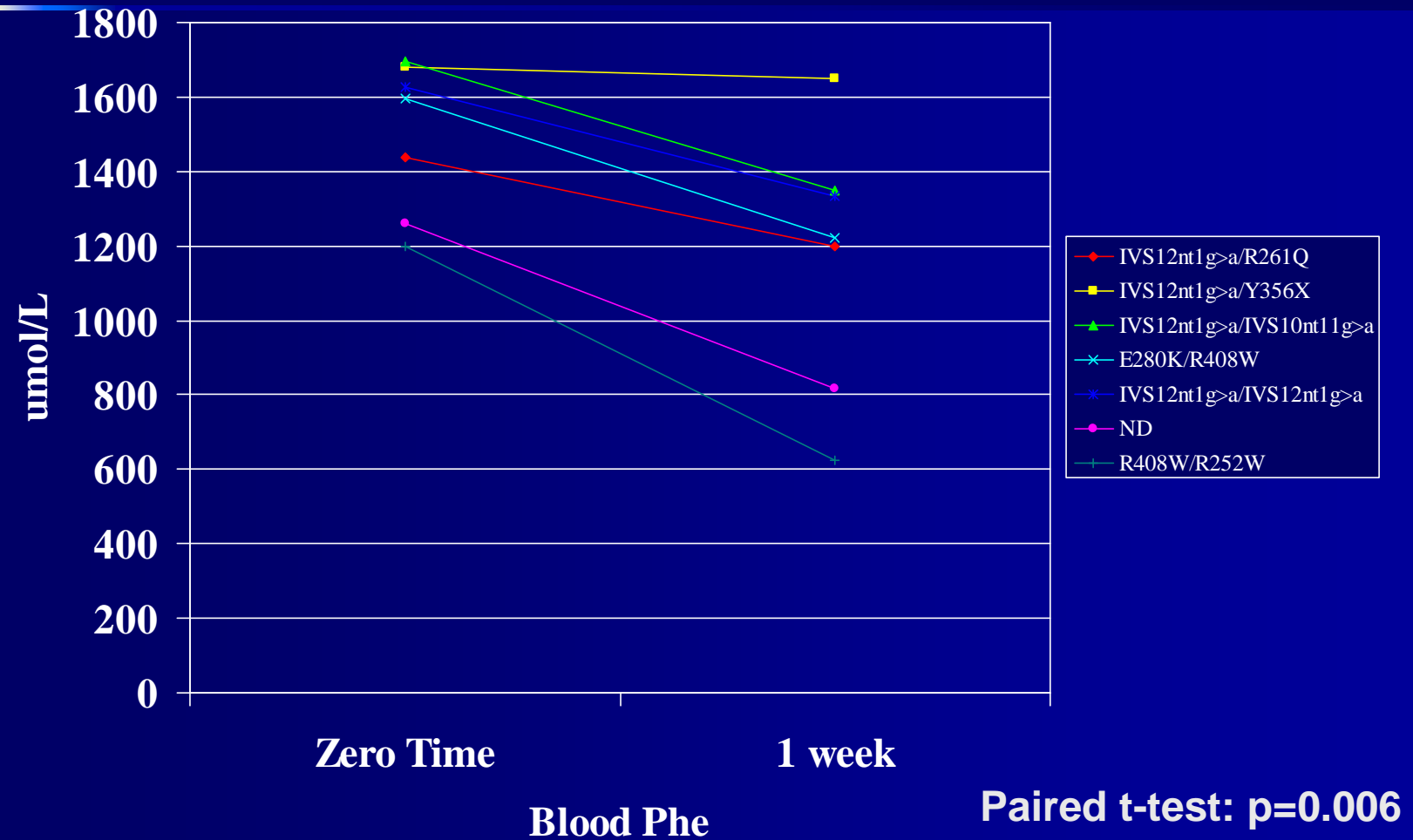


Figure 2. Blood Phe Response to 1.0 g/kg NeoPhe in Patients with PKU



CONCLUSIONS:

- For the first time mixture of LNAA can lower blood phenylalanine
- Using NeoPhe avoids lysine deficiency and negative nitrogen balance
- NeoPhe offers new options to treat PKU
- Diet can be more liberal with NeoPhe
- Still blood phe- levels need to be controlled